Innovative Medicines Initiative

Patient involvement in the Innovative Medicines Initiative

Results of a questionnaire and interview study

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Contents

Abbreviations

ABPI	Association of the British Pharmaceutical Industry
APMAR	Associazione Persone con Malattie Reumatiche
BioVacSafe	Biomarkers for Enhanced Vaccine Safety
BSR	British Society of Rheumatology
BSPAR	British Society for Paediatric and Adolescent Rheumatology
BTCURE	Be The Cure
CEO	Chief Executive Officer
COMBACTE	Combating Bacterial Resistance in Europe
COMPACT	Collaboration on the optimisation of macromolecular pharmaceutical access to cellular targets
DDMoRe	Drug Disease Model Resources
EHR4CR	Electronic Health Records Systems for Clinical Research
EC	European Commission
ECPC	European Cancer Patient Coalitions
EFA	European Federation of Allergy and Airways Diseases Patients Associations
EFPIA	European Federation of Pharmaceutical Industry Associations
EHC	European Haemophilia Consortium
ELF	European Lead Factory
EMA	European Medicines Agency
EMIF	European Medical Information Framework
EMTRAIN	European Medicines Research Training Network
EP	European Parliament
EU	European Union
EU-AIMS	European Autism Interventions
EULAR	European League Against Rheumatism
EUReMS	European Register for Multiple Sclerosis
EUPATI	European Patient's Academy on Therapeutic Innovation
EURORDIS	European Organization of Rare Diseases
FESCA	Federation of European Scleroderma Associations
FP7	EU's 7th Framework Programme for Research
GAVI	Global Alliance for Vaccines and Immunisation
GP	General Practitioner
HTA	Health Technology Assessment
IDF	International Diabetes Federation
IMI	Innovative Medicines Initiative
INSERM	Institut National de la Santé et de la Recherche Médicale
K4DD	Kinetics for Drug Discovery
LSE	London School of Economics
MIP-DILI	Mechanism-Based Integrated Systems for the Prediction of Drug-Induced Liver Injury
MEP	Member of the European Parliament
MTRG	Medical Technology Research Group, LSE
NICE	National Institute for Health and Care Excellence
NHS	National Health Service
NGO	Non-Governmental Organisation

Open PHACTS	The Open Pharmacological Concepts Triple Store
ORBITO	Oral biopharmaceutics tools
SAFE-T	Safer and Faster Evidence-based Translation
PARE	EULAR Standing Committee for People with Arthritis/Rheumatism in Europe
PD	Parkinson's Disease
PFI	Private Finance Initiative
PPP	Public-Private Partnership
R&D	Research & Development
TB	Tuberculosis

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Executive summary

Background

The involvement of patients in health policy and clinical research has gained prominence in recent years, with central agencies such as the European Medicines Agency (EMA) and National Institute of Health and Care Excellence (NICE) adopting formal procedures and structures for patient involvement. The benefits of involving patients include policymaking and research that is more responsive to need, as well as more democratic and acceptable to stakeholders. The aim of this work is to assess the potential for patient involvement in the work of the Innovative Medicines Initiative (IMI) and to make recommendations for how this can be achieved.

Methods

Respondents from patient organisations at national and international levels were invited to participate in a questionnaire study assessing their current knowledge of IMI and PPP's more generally; attitudes towards the utility of PPP's as a public investment priority; the relevance of the work of IMI to patients; experience of and expectations for patient involvement in research; and attitudes towards the pharmaceutical industry.

The questionnaire was initially developed with feedback from a patient involvement expert, piloted among a small number of individuals, and subsequently rolled out on a large scale. A total of 472 individuals were invited to participate. Subsequent to the questionnaire, in-depth interviews were undertaken with representatives from five organisations to further enlighten the results of the questionnaire.

Results

A total of 159 respondents completed the questionnaire (34% response rate), of which 77% and 23% represented national and international/European level organisations, respectively. The most frequent therapeutic areas accounted for 65% of responses and included cancers, bleeding disorders, neurological disorders, rheumatic disorders, rare diseases, autoimmune diseases and stroke. A large proportion (64%) could be categorised as senior representatives of their organisations, including president, chief executive, chairman, board member etc.

The most common sources of research information were conferences and professional gatherings, clinicians and health professionals and from newsletters, websites or colleagues from other organisations. PPP's were relatively well known in the sample, and IMI was the partnership most frequently given as an example, followed by specific IMI projects such as EUPATI and BTCure. The most well-known IMI project was EUPATI with 31% recognising the acronym, followed by EUROPAIN (8%).

Respondents who had previously heard of IMI (39%) with some exceptions knew what IMI was, and had most commonly heard of IMI through conferences, meetings or news and through other patient organisations who had collaborated with IMI. Most respondents thought IMI played an important role in encouraging research on innovative medicines, felt IMI's research was relevant to patients and families of their organisation, and were positive about becoming involved in IMI activities. A majority (68%) stated education and training activities to enhance patient's understanding of R&D

would be valuable to their organisation, as would being able to give input to the IMI research agenda (54%).

Many organisations stated they had no interactions with EU institutions (39%), while almost a third attended conferences organised by EU institutions on a regular basis and a quarter were engaged in lobbying activities.

Knowledge of the pharmaceutical industry was fairly limited, with only 42% correctly identifying the role of EFPIA, and only 31% estimating the cost of developing a novel pharmaceutical at the generally accepted level of €600-800 million. Only 9% correctly answered that funding for pharmaceutical R&D was equally split between the private industry and public sector.

The majority (89%) of respondents thought patients could contribute to R&D by helping researchers understand which clinical benefits are important to patients, and by providing input on the design of clinical trials (65%) and disseminating results of research (59%). On previous involvement, one third had been involved in distributing research, and one third had consulted with researchers on which clinical benefits are important to patients. Additionally, one third had not been involved in any research. Potential barriers were most importantly that patient knowledge was perceived as less important than that of clinicians (65%) or that of scientists (61%), and that patients were not given opportunities to become involved by researchers (64%).

There was no clear favour towards investing in PPP's as a public priority when compared with supporting investment outside the health system, investing in prevention or making existing therapies more widely available. These strategies were generally equally favoured, though the majority (68%) thought it was important to invest public money in PPP's. Most respondents (73%) also agreed that insufficient funds would be available for rare disease research if not supported by PPP's.

There was a tendency for respondents to think more positively of the pharmaceutical industry (35%) considering their involvement in PPP's, and to think the industry was more concerned about patient welfare (35%), though perceptions on profit motive were largely unchanged. The highest level of agreement was with the industry being concerned about their public perception while wanting to make a positive difference to patients at the same time (42% agreed).

In the final part of the questionnaire, four overall themes emerged from the comments provided by respondents: there should be direct communication and partnership between IMI and patient organisations; the information on participation must be clear and accessible; patient organisations require education and training to be able to participate successfully; and there is a need for a centralised platform to facilitate patient involvement.

Shedding further light on these issues, participants in the in-depth interviews pointed out that the climate for patient involvement was changing, much due to the changing nature of the doctor-patient relationship. While all informants agreed patients possessed valuable information and experience that would contribute to clinical research, it was also pointed out that lack of knowledge among patient organisations as to how to participate was a significant barrier to involvement. Furthermore, informants emphasised the importance of approaching and engaging patient

organisations at the grassroots level, stating that information must be given as close to the patient as possible to be effective.

When cross-comparing answers from different questions, several correlations were noted: respondents from EU level organisations were twice as likely to have heard of IMI (60%) than respondents from national organisations (34%). Respondents who thought IMI's work was important generally also thought it was relevant to their organisation, and further were more likely to be interested in collaborating with IMI. In general, respondents thought it was important to invest public money in PPP's, and there was a tendency for respondents who were familiar with IMI to attribute higher importance to investment in PPP's. Respondents who correctly estimated the cost of developing a new drug at €600-800m also tended to agree more that PPP's were necessary to stimulate R&D for conditions with small commercial markets.

Knowledge of IMI was significantly associated with more positive attitudes towards the industry's involvement in PPP initiatives, and also with a stronger perception that the industry is concerned about patient welfare. Respondents familiar with IMI also agreed to a greater extent that the industry's main reason for engaging in PPP's was to improve public perception as well as making a positive difference to patients.

Finally, the proportion of respondents already involved in various R&D aspects was without exception markedly lower than the proportion of patients judging those aspects important. For example, while almost all participants felt patient organisations should play a stronger role in helping researchers understand clinical benefits important to patients, only one third had already participated in this activity.

Recommendations for IMI

Based on the findings of this report, three recommendations are given:

- ***Awareness of IMI:*** In order to gain awareness among patient organisations at national and grassroots level, IMI must aim at providing information in both a harmonised and highly contextualised manner, and approach patient organisations directly and at the local level.

- ***Involvement in research:*** IMI should aim at better involving patients by focusing on overcoming the barriers. IMI should try to understand patient organisations using a bottom up approach, focusing on grassroots organisations in different geographical contexts. It should also aim to facilitate education and training for those wanting to participate. Finally, IMI should aim to encourage research environments involving patient organisations to be aware of the "medical talk" and discourse used in partnership with patient organisations that might be problematic.

- ***Perception of PPPs:*** If insights into the perception of the pharmaceutical industry are to be investigated, other areas should be explored such as corporate social responsibility, reliability and integrity in order to provide a more complete picture.

List of figures and tables

Figures

Tables

1. Introduction

Recent years have seen increasing involvement of patients in decisions concerning clinical research, health policy and reimbursement (Kaye et al., 2012; Stacey, et al., 2008; Elberse, Pittens, Buning, & Broerse, 2012). Examples of such engagement include patient representatives at the Management Board of European Medicines Agency (EMA) and in advisory committees within the National Institute of Health and Clinical Excellence (NICE) in the United Kingdom. The benefits of involving patients are developments in care, policy and research that are more responsive to need, better acceptance of the decisions made and a more democratic process (Elberse, Pittens, Buning, & Broerse, 2012). But despite increased attention to the societal relevance of research, inputs from patients are often considered "subjective" (Elberse, et al., 2012). Although patient surveys indicate that patients want and expect to be involved in making health related decisions (Stacey, Samant, & Bennett, 2008), there is a lack of awareness among patients and their associations on how to proceed.

This report was commissioned by the Innovative Medicines Initiative (IMI) to assess the potential for patient involvement in the work of IMI. The present report presents the second work package of the study, comprising two key components, notably the large scale roll-out of the previously piloted patient organisation questionnaire and a number of in-depth interviews with high profile patient advocates carried out by the Medical Technology Research Group at the London School of Economics. The report addresses three objectives, as follows:

- First, the level of awareness of IMI among patient organisations and the reasons that may explain awareness or lack thereof;
- Second, the perception of the pharmaceutical industry following involvement in public private partnerships (PPP);
- Third, the extent of patient and patient organisation involvement in IMI and its activities and suggestions for likely improvements.

The report is structured in three parts. Part one provides a brief introduction to the field of patient involvement and the report in general. Part two presents the methodology employed. Part three gives an overview of the results of the questionnaire and interviews, and part four a discussion. Finally, some concluding remarks on the study and its results are given, including implication and recommendations for IMI's future work. The questionnaire, the interview guide and the interview transcripts are provided in the appendix.

2. Methods

2.1. The Questionnaire

The methods employed for the questionnaire design, piloting and sampling are laid out below.

2.1.1. Design and piloting

The cross-sectional questionnaire was developed in two phases. In phase one, a pilot questionnaire was designed and validated. This was carried out in three steps. First, the question items and wording were discussed with an expert in patient involvement. In response to the expert feedback, a number of minor improvements were made to the questionnaire. This included simplifying the wording of some questions to better suit lay audiences, and changes to the response alternatives offered to the respondents.

Second, during piloting where hard-copy questionnaires were filled out by respondents in the presence of a consultant, respondents were asked to identify parts of the questionnaire they found problematic. While the majority of respondents felt the questionnaire was interesting, a few minor issues were raised. Some respondents felt the questionnaire was too long, while another respondent felt some questions were phrased in a leading manner, and should be more neutral.

Third, after piloting the validity of the questions was assessed by comparing responses to questions considered to be related. The narrative of responses collected was examined and the inconsistencies caused by the questionnaire were identified.

In phase two, the questionnaire was revised based on the process of validation. Finally, the revised questionnaire was launched online for the large-scale rollout.

2.1.2. Questionnaire description

The final questionnaire was structured in six parts. Part one contained questions about respondents' baseline characteristics, such as the country and disease area they work in as well as their role within the organisation. In part two, respondents were asked questions about their awareness of IMI and PPP's and their interest in participating in IMI's activities. In part three, questions were asked about respondents' knowledge of and interaction with the European Union. In part four, questions were asked about respondents' knowledge of pharmaceutical actors and research. In part five, respondents' opinions on investment priorities were gauged. Finally, part six contained questions on respondents' perception of the pharmaceutical industry and its involvement in PPP's. For a complete version of the questionnaire, please see Appendix 1: Questionnaire.

2.2. Sampling

Purposive followed by snowball sampling was employed. Based on the fourth edition of the European Patient Group Directory, as well as the MTRG, Patient Academy and LSE Health's extensive network of partners both at the national and international level in Europe, patient organisations and individuals were invited by email to participate in the online questionnaire. In total 236 respondents were directly invited to participate. Further to this, invitees were requested to identify colleagues within or outside their organisation to be included in the sample. This was done in two ways: either the individual forwarded the invitation and provided the researchers with the number of people who received the invitation (for respondents concerned about privacy), or the individual provided MTRG

with contact details for the invitation to be sent out directly. This process resulted in an additional 254 respondents invited.

2.3. Data collection from the questionnaire survey

Data was collected online between 12th June and 11th August 2013. Invitations to participate were sent out to a total of 490 unique email addresses. Of these, 372 invitations were sent out directly from MTRG and 118 were sent out via respondent organisations. Of the 372 invitations, contact details for 74 invitations were provided by nine respondents, and a further 44 individuals received the invitation in a member's bulletin[1].

The first invitations were sent out on 12th June, and reminders were sent out on 5th July and 5th August. Invitations were also sent out continuously between the 12th June and 5th July, as new potential participants were identified.

2.4. In-depth interviews

Subsequent to the questionnaire, in-depth interviews were undertaken with key informants to further enlighten the results of the questionnaire. This allowed for elaboration on issues assessed in the standardised questionnaire, and gave participants the opportunity to express themselves more freely.

2.4.1. Sampling

Purposive and snowball sampling were used as sampling methods. Individuals receiving the invitation to participate in the questionnaire were invited to make contact if they wished to participate in the interview round. Two of the interviewees were recommended by another questionnaire participant.

2.4.2. Data collection

Interviews were conducted in July and August 2013 by telephone following an interview guide consisting of six questions. Interviews lasted between 15 and 20 minutes. In order to ensure the validity of the transcripts, a member check was conducted in which interviewees were given the opportunity to comment on transcripts. The interview guide and transcripts are provided in the appendix.

2.5. Analysis

The analysis of the questionnaire data was performed using descriptive and inferential statistics, and structured to address the study objectives and research questions, namely: a) the awareness of IMI, b) the perception of the pharmaceutical industry following PPP participation, and c) the involvement in IMI. The statistical package STATA 10E was used for all statistical analyses using a 5% significance level. Chi-square tests were used to explore associations between categorical variables, Mann-Whitney U was used to test for differences in ordinal variables between two subgroups, and Spearman Rank (rho) was used to test for correlations between two ordinal variables.

The in-depth interview data was analysed following an inter-case thematic analysis, adopting the framework developed by Miles and Huberman (1994). The framework describes qualitative data

[1] The number of respondents opening the email was tracked by the sender.

analysis in terms of three phases: a) data reduction, b) data display and c) conclusion drawing and verification.

a) Data reduction: In order for the qualitative data to be meaningful, it was organised to allow a process of selecting and abstracting. In phase one, the transcripts were read by the analyst to become familiar with the data and pay attention to specific patterns. Second, the transcripts were grouped by questions and an initial coding into conceptual categories was generated to see where patterns occurred. Third, codes were given meaning and combined into over-arching themes using colour coding, and the data was re-read to see how the themes supported the data.

b) Data display: The second phase aimed to compress the results of the data reduction in a way that allowed conclusions to be drawn, providing a new way of arranging and thinking about the data. An illustrative matrix was developed to synthesise the information provided.

c) Conclusion drawing and verification: Conclusions were developed and verified by a re-examination of the data. Themes and interpretations of the data were tested by looking for competing themes and reviewing outliers.

2.6. Limitations

Several limitations to the present report should be noted. First, both the roll-out of the questionnaire and the interview sessions may have been affected by the timing, as Europeans are generally on holiday in June, July and August, which could help explain the response rate. Second, online surveys are known to have low response rates, as email recipients receive numerous requests and do not feel obliged to follow up. Third, numbers for referral of invitations (the snowball sampling) are based on the information provided by study participants, meaning the researchers do not have direct control of how many actually received the invitation. Fourth, statistical analysis on a sample of only 159 observations should be interpreted as indicative and conclusions should be handled with caution, except where the level of significance is very high.

3. Results

A total of 159 respondents completed the questionnaire, resulting in a response rate of 34%. While some questions were targeted to all respondents, others were intended only for respondents reporting familiarity with PPP's and IMI, which is reflected in the denominator of each question.

In some questions respondents were invited to provide comments or non-standard answers by selecting the "Other" option. These comments are summarised in the text, and a comprehensive list of comments can be found in Appendix 2: Supplement to questionnaire.

3.1. Respondent characteristics

In the first section, respondents were asked to provide their country, therapeutic area and role within their organisation. Results are listed by frequency (Table 3.1).

77% (122 of 159) respondents represented national organisations, while 23% (36 of 159) represented an organisation at European or international level. One respondent did not provide country/location. The latter group also included respondents listing 5 or more countries. Among the respondents representing national organisations, five respondents (3%) stated that they also represented their organisation at EU level.

Diseases and disease areas were grouped together to make meaningful categories[2]. The seven most frequent disease areas accounted for 65% of the responses (including cancers [26 of 159, 16%], neurological disorders [22 of 159, 14%], bleeding disorders [16 of 159, 10%], rheumatic disorders [13 of 159, 8%], rare diseases [12 of 159, 7%], autoimmune diseases [11 of 159, 7%] and stroke [11 of 159, 7%].

The most frequent roles included president of the organisation (28 of 159, 18%), chief executive (22 of 159, 14%), chairman (16 of 159, 10%) and "other" (46 of 159, 29%) which included a range of roles including vice presidents, vice chief executives, information officers etc. The majority of respondents could be identified as "senior" within their organisation [102 of 159, 64%), including president, chief executive, chairman, board member and related titles.

[2] Airway diseases (including asthma and COPD); Autoimmune diseases (including Psoriasis and Psoriatic Arthritis, SLE, Lupus, Raynaud's and Scleroderma); Bleeding disorders (including haemophilia and myelodysplastic syndrome); Cancer (including lung, kidney, multiple myeloma, cervical, lymphoma, chronic myeloid leukaemia and rare cancers); Chronic inflammatory diseases (including Ankylosing spondylitis) Chronic inflammatory diseases (including Ankylosing spondylitis); Genetic disorders (icnluding Down syndrome and Alpha-1 Antitripsin Deficiency); Mental illnesses (including schizophrenia, bipolar disorder and depression); Neurological disorders (including Dystonia, epilepsy and parkinson's disease); Pelvic floor and urinary tract diseases (including pain in pelvis); Rare diseases (including cystic fibrosis); Stroke (including conditions requiring anticoagulation therapy). One respondent claimed working in lung and heart transplant, which was not seen as a disease or therapeutic area but rather reflecting a procedure, and was therefore not included in this list.

Table 3.1 Respondent characteristics.

Country (n=158)		Therapeutic area (n=171[3])		Respondent's role (n=159)	
United Kingdom	33	Cancers	26	Other[4]	46
Europe	27	Neurological disorders	22	President	28
France	11	Bleeding disorders	16	Chief Executive	22
International	9	Rheumatic disorders	13	Chairman	16
Denmark	7	Rare diseases	12	Board Member	14
Greece	7	Autoimmune diseases	11	Member (patient)	13
Hungary	7	Stroke	11	Secretary General	7
Spain	7	Multiple sclerosis	6	Advisor	4
Germany	6	Allergy	5	Consultant	3
Romania	6	Airway diseases	4	Member (carer)	2
Italy	5	Asthma	3	Member (relative)	2
The Netherlands	5	Cardiovascular diseases	3	Policy Director	2
Ireland	3	Mental illnesses	3		
Malta	3	Muscular diseases	3		
Bulgaria	2	Chronic inflammatory diseases	2		
Cyprus	2	Chronic pain syndrome	2		
Israel	2	Diabetes	2		
Lithuania	2	Genetic disorders	2		
Sweden	2	Migraine	2		
Austria	1	Pelvic floor and urinary tract diseases	2		
Belgium	1	Retinal degenerative diseases	2		
Croatia	1	Bone diseases (osteoporosis)	2		
Estonia	1	Endometriosis	1		
Finland	1	Heart rhythm disorders	1		
Latvia	1	Immune-mediated inflammatory disorder	1		
Macedonia	1	Metabolic disorders	1		
Norway	1	Movement disorders	1		
Poland	1	Primary immunodeficiency	1		
Portugal	1				
Serbia	1				
Slovakia	1				

3.2. Sources of information about R&D

There were several significant sources of information used by respondents to keep updated about relevant research and development, most importantly conferences and other professional gatherings (136 of 159, 86%), clinicians and health professionals (113 of 159, 71%) and from

[3] Some respondents worked in more than one disease area.
[4] "Other" included a range of roles, including vice presidents, vice chief executives and information officers.

newsletters, websites or colleagues from other organisations (103 of 159, 65%) as shown in Table 3.2.

Table 3.2 Sources of R&D information.

What are your three most important sources of information about research and development relevant to your organisation? Select all that apply	
Conferences and other professional gatherings	136 (86%)
Clinicians/healthcare professionals	113 (71%)
Newsletters, websites or colleagues from other organisations	103 (65%)
Scientific literature	99 (62%)
Newsletter, website or colleagues within my own organisation	78 (49%)
Industry and industry-related newsletters or websites	54 (34%)
Social media (Facebook, Twitter, blogs etc.)	53 (33%)
Specialised bulletins	47 (30%)
Newspapers or television	36 (23%)
Other	8 (5%)

3.3. Familiarity with public private partnerships (PPP's)

A total of 61% (97 of 159) of respondents had previously heard of the term Public Private Partnerships (PPP's), while 27% (43 of 159) had not, and 12% (19 of 159) were not sure.

When asked to list any PPP's they knew of, IMI was the PPP most commonly mentioned by respondents (19 cases, 12%), with two IMI projects also mentioned including EUPATI (5 cases, 3%) and BeTCure [*sic*, BTCure] (2 cases, 1%) as shown in Table 3.3. For the comprehensive list of answers, please see Appendix 2: Supplement to questionnaire.

Table 3.3 Examples of PPP's known to respondents.

Can you think of and name any examples of a public-private partnership?
IMI (19 cases, 12%)
EUPATI (5 cases, 3%)
Autocure (2 cases, 1%)
BeTCure (2 cases, 1%)
Global Fund (TB, Malaria, AIDS), GAVI (1 case, 0.6%)
Drugs for Neglected Diseases Initiative (1 case, 0.6%)

3.4. Knowledge and perception of IMI

Respondents were asked if they had heard of IMI, to which 62 of 159 responded "yes" (39%), while 87 (55%) responded "no" and the remaining 10 (6%) were unsure. Respondents indicating no knowledge of IMI were excluded from answering the following six questions, leaving 72 potential respondents, of which some did not respond: 55 of 67 respondents (82%) correctly identified IMI as *"A partnership between the European Union and European pharmaceutical companies to encourage development of innovative medicines",* while 6 of 67 (9%) were unsure about what the IMI was (Table 3.4). The most common cause of familiarity with IMI was through conferences, meetings or

news (28 of 67, 42%) followed by knowing other patient organisations that have collaborated with IMI (12 of 67, 18%) as shown in Table 3.5. Some respondents (8 of 67, 12%) had already collaborated with IMI or had been contacted directly by IMI.

Table 3.4 Knowledge about IMI.

Based on your current knowledge of IMI, would you describe IMI as	
A partnership between the European Union and European pharmaceutical companies to encourage development of innovative medicines	55 (82%)
Not sure	6 (9%)
A global partnership of charitable organisations with an interest in health	3 (5%)
A Geneva-based organisation that coordinates public-private partnerships between national governments and the pharmaceutical industry	3 (5%)
A private network for North American pharmaceutical companies to share the risk of developing innovative medicines	0 (0%)

Table 3.5 Sources of knowledge on IMI.

How would you best explain why you have heard about IMI?	
I am familiar with IMI more generally through conferences, meetings or news	28 (42%)
I know of other patient organisations that have collaborated with IMI	12 (18%)
Not sure	8 (12%)
My organisation has collaborated with IMI	8 (12%)
My organisation has been contacted by IMI	7 (10%)
Other	4 (6%)

Respondents generally perceived IMI as playing an important role in encouraging research on the development of innovative medicines, replying mostly “very important” (34 of 69, 49%) or “somewhat important” (26 of 69, 38%) as shown in Figure 3.1. A majority (52 of 69, 76%) felt IMI’s research was “very relevant” or “moderately relevant” to patients and their families, and also (56 of 69, 84%) stated a positive attitude towards becoming involved in IMI activities (Figure 3.1).

The majority (49 of 72, 68%) stated that involvement in education and training activities to enhance patients' understanding of R&D would be valuable to their organisation, as would being able to give input to the IMI research agenda (39 of 72, 54%). Many respondents (34 of 72, 47%) saw value in helping to decide which medical areas IMI should focus on (Table 3.6). One respondent remarked that their organisation would not have the resources to participate, and another that it was not really the industry’s task to provide education and training. The full list of comments can be found in Appendix 2: Supplement to questionnaire

Figure 3.1 Responses regarding the activities, importance and potential for participation with IMI.

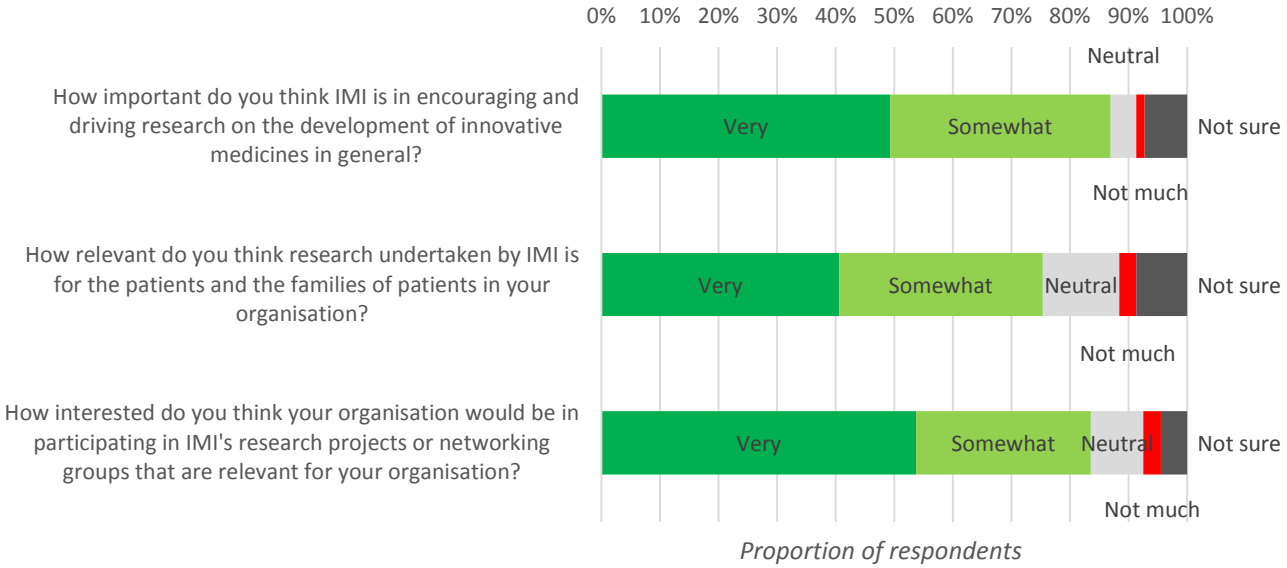


Table 3.6 Preferences on the potential mode of involvement with IMI.

Which type of involvement do you think is likely to be valuable for your organisation if offered to participate in IMI's activities? Select all that apply	
Being involved in IMI's education and training projects to enhance patients' understanding of clinical research and development	49 (68%)
Being able to give input to IMI's research agenda	39 (54%)
Helping to decide which medical areas IMI should focus on	34 (47%)
Being directly involved with the companies carrying out research	28 (39%)
Being involved in projects related to the testing and monitoring of aspects related to the safety and efficacy of pharmaceuticals and diagnostics	28 (39%)
Other	6 (8%)

3.5. Familiarity with IMI projects

Since respondents may have been aware of IMI projects without realising the affiliation, all respondents were asked to indicate which of a list of IMI projects they were familiar with. Almost one third (49 of 159, 31%) had heard of EUPATI, 13 respondents (8%) had heard of EUROPAIN and smaller numbers had heard of additional IMI projects as shown in Table 3.7.

Table 3.7 IMI projects familiar to respondents.

Please indicate which (if any) of IMI's projects you are familiar with/have heard of	
EUPATI	49 (31%)
EUROPAIN	13 (8%)
Pharmatrain	7 (4%)
BTCure	6 (4%)
U-BIOPRED	6 (4%)
NEWMEDS	5 (3%)
DIRECT	4 (3%)
PreDiCT-TB	4 (3%)
SUMMIT	3 (2%)
EU-AIMS	2 (1%)
Eu2P	2 (1%)
Onco Track	2 (1%)
Pharma-Cog	2 (1%)
SafeSciMET	2 (1%)
COMBACT	1 (1%)
COMPACTE	1 (1%)
EHR4CR	1 (1%)
eTRIKS	1 (1%)
IMIDIA	1 (1%)
RAPP-ID	1 (1%)
TRANSLOCATION	1 (1%)

3.6. Knowledge of and interaction with EU institutions

A general question on the role of the European Commission was asked to gauge respondents' knowledge of the EU system in general (Table 3.8). One third (54 of 159, 34%) correctly answered "*The EC works like a national government, with departments running various aspects of the EU*", with slightly more (57 of 159, 36%) mistaking the EC for "*The EC is an assembly of the European heads of state/government responsible for defining the general political direction and priorities of the EU*".

Table 3.8 Knowledge about the European Commission (EC).

Which of the following statements about the European Commission (EC) is the most appropriate?	
The EC is an assembly of the European heads of state/government responsible for defining the general political direction and priorities of the EU	57 (36%)
The EC works like a national government, with departments running various aspects of the EU	54 (34%)
The EC works like a national parliament and votes on European legislation	24 (15%)
Not sure	24 (15%)

When asked about their organisations mode of interaction with EU bodies, 39% (62 of 159) stated their organisation currently had no interaction with the EU institutions, while 29% (46 or 159) attended conferences organised by the EU institutions on a regular basis, and 25% (40 of 159) were engaged in lobbying activities (Table 3.9). Under the category of "other" activities, respondents reported, for example, participating in meetings with the European Commission and interaction through membership or collaboration with umbrella organisations within their disease area. For a comprehensive list of specifications, please see Appendix 2: Supplement to questionnaire.

Table 3.9 Modes of interaction between patient organisations and European bodies.

Does your organisation have any interaction with the EU institutions (the EU Parliament, the EU Commission, the European Council)? Select all that apply	
No interaction currently	62 (39%)
My organisation attends conferences organised by the EU institutions on a regular basis	46 (29%)
Lobbying	40 (25%)
My organisation participates in research funded by the EU institutions	30 (19%)
Other	19 (12%)
Permanent representation/office in Brussels	14 (9%)
Not sure	12 (8%)

3.7. Knowledge of pharmaceutical actors and research

Questions assessing the qualitative knowledge of respondents on the actors within the pharmaceutical research sphere revealed that 42% (66 of 159) could correctly identify the European Federation of Pharmaceutical Industries and Associations (EFPIA) as "*A private industry association representing national pharmaceutical industry associations in Europe*". The remaining respondents described EFPIA as a PPP (33 of 159, 21%), private research organisation (6 of 159, 4%) or answered "not sure" (54 of 159, 34%) (Table 3.10).

Table 3.10 Knowledge of European Federation of Pharmaceutical Industry and Assoc. (EFPIA).

The European Federation of Pharmaceutical Industries and Associations (EFPIA) is a partner of IMI. Would you describe EFPIA as (select the most appropriate option)	
A private industry association representing national pharmaceutical industry associations in Europe	66 (42%)
Not sure	54 (34%)
A public-private partnership to increase collaboration within the European pharmaceutical industry	33 (21%)
A private research organisation which pools resources from European pharmaceutical companies	6 (4%)

When asked how high the cost of developing a new pharmaceutical is, one third (50 of 159, 31%) answered the highest possible option of €600-800 million (Table 3.11). While the exact figure is subject to debate in the scientific community, a generally accepted figure is around the US$ 1 billion

mark (€600-800 million). Significant proportions of respondents were either not sure (44 of 159, 28%), or answered €300-450 million (41 of 159, 26%) or lower (24 of 159, 15%).

Table 3.11 Knowledge of development costs of new pharmaceuticals.

What do you think is the approximate average cost of developing and marketing a new pharmaceutical product?	
€600-800 million	50 (31%)
Not sure	44 (28%)
€300-450 million	41 (26%)
Approx. €200million	19 (12%)
Less than €80million	5 (3%)

A large majority of respondents answered the main funder of pharmaceutical R&D was the pharmaceutical industry (Table 3.12) (123 of 159, 77%). A small minority (15 of 159, 9%) answered equal contributions from industry and the public sector (Table 3.12). According to official figures, the majority of pharmaceutical R&D funding is in fact split in equal proportions between industry and the public sector, with smaller contributions from other sectors.

Table 3.12 Perception of financial arrangements for R&D on pharmaceuticals.

Who do you think contributes the most money to global R&D on pharmaceuticals?	
The pharmaceutical industry	123 (77%)
Not sure	16 (10%)
Approximately equal contribution from governments and the pharmaceutical industry	15 (9%)
Non-Governmental Organisations, foundations and charities	3 (2%)
National and supranational governments	2 (1%)

3.8. Experience of and interest in patient involvement in pharmaceutical R&D

A large majority of respondents answered patients should participate in pharmaceutical R&D by helping researchers to understand which clinical benefits are important to patients (142 of 159, 89%), and by participating in the design of clinical trials (103 of 159, 65%). Almost equally important was the patients role in distributing the results of research (94 of 159, 59%). Approximately half of respondents felt a stronger role should be played by patients in deciding which medical conditions should be researched (82 of 159, 52%), and that patients should help companies make their products available in their national health systems (81 of 159, 51%) (Table 3.13).

Under the "other" response field, one respondent pointed out that some patient organisations are more politically active and effective as pressure groups than others, and that it would be unfair to leave decisions on development to the loudest voice. Two respondents mentioned that partnerships would be a useful approach, while others suggested patient organisations could help fund research or be involved through clinical trials, though it was also pointed out that participation in clinical trial

design tended to be limited and non-technical. One respondent noted patients could help ensure drugs are marketed at a price that is fair both to the industry and to society. Finally, one respondent pointed out that patient organisation not always, if ever, had access to research, making it difficult to distribute it. A comprehensive list of comments can be found in Appendix 2: Supplement to questionnaire.

Table 3.13 Potential roles for patient involvement in R&D.

Do you think patients or patient organisations should play a stronger role in pharmaceutical R&D? Select all that apply	
Yes, by helping researchers understand which clinical benefits are important to patients	142 (89%)
Yes, by participating in the design of clinical trials	103 (65%)
Yes, by distributing the results of research	94 (59%)
Yes, by helping companies make their products available through national health systems	82 (52%)
Yes, by helping to decide for which medical conditions technologies should be developed	81 (51%)
Other	9 (6%)
Not sure	3 (2%)
No	0 (0%)

When asked whether the respondent's organisations had already participated in any pharmaceutical related R&D activities (Table 3.14), more than one third (57 of 159, 36%) stated they had been involved in distributing research, and a similar proportion (53 of 159, 33%) had been involved in consulting researchers on what clinical benefits are important to patients. On the other hand one third (53 of 159, 33%) had not been involved in any pharmaceutical R&D related activities.

Respondents elaborating under "Other" added that while they themselves had not participated, other members had. One respondent added participation in clinical trials, and another indicated that plans for participation were in the making. Others answered they were already funding research, were involved in patient recruitment, dissemination of research results, or had given presentations to researchers about their condition. A comprehensive list of comments can be found in Appendix 2: Supplement to questionnaire.

Table 3.14 Previous participation in pharmaceutical R&D.

Has your organisation participated in any pharmaceutical R&D related activities?	
Yes, by distributing the results of research	57 (36%)
No	53 (33%)
Yes, by helping researchers understand which clinical benefits are important to patients	53 (33%)
Yes, by helping companies make their products available through national health systems	30 (19%)
Yes, by participating in the design of clinical trials	26 (16%)
Not sure	24 (15%)
Yes, by helping to decide for which medical conditions technologies should be developed	14 (9%)
Other	13 (8%)

The most prominent reason given as barrier to patient involvement in R&D was that patient knowledge was perceived as less important than that of clinicians (104 of 159, 65%) and that of scientists (97 of 159, 61%) and that patients were not given the opportunity to become involved by those carrying out research (101 of 159, 64%). Significant proportions also indicated patients did not know what research was being carried out (92 of 159, 60%) or how to approach researchers to become involved (89 of 159, 56%) (Table 3.15).

When asked to elaborate under "Other", several respondents pointed out that education and training of patient advocates was needed. For example, one respondent raised the issue of lack of training offered to patients to fully participate, and that patient organisations often were approached very late in the process. One other respondent pointed out the information asymmetry between the two sides resulting in communication difficulties. One respondent made the point that although patients' views were listened to they were not taken into account at the moment when decisions were made. Yet another respondent claimed that time and resource constraints were important barriers as patient organisations did not have the means to fund employees for this type of participation. Interestingly, one respondent noted that patient organisations themselves needed to promote research as a "full part of the patient's issue and solution". A comprehensive list of comments can be found in Appendix 2: Supplement to questionnaire.

Table 3.15 Barriers to patient involvement in R&D.

What do you think are the main challenges for successful involvement of patient and patient organisations in R&D?[5]	
Patient's knowledge is perceived as being less important than knowledge from clinicians	104 (65%)
Patients are not given the opportunity to become involved by those undertaking research	101 (64%)
Patient's knowledge is perceived as being less important than knowledge from scientists	97 (61%)
Patients don't know what research is being carried out	92 (60%)
Patients are not sure how to approach researches to become involved	89 (56%)
It is difficult for a small group of patients to represent the opinions of all patients with the same medical condition	67 (42%)
Other	18 (11%)
Not sure	6 (4%)

3.9. Opinion about investment priorities

To gauge the utility of PPP's as an investment priority relative to other health related public priorities, respondents were asked to indicate how they preferred taxpayer money to be spent (Table 3.16). Although this to some extent is a collection of partisan views, there was almost equal support for four strategies: supporting investment outside the health system, investing in prevention (each at 34 of 159, 21%), as well as investing in research for new pharmaceuticals, and making already existing therapies more available to patients by increasing the funding of these (each at 31 of 159, 19%).

When asked to elaborate under "other", several respondents held that all the strategies were required and could not prioritised, while another claimed that a mix of the listed strategies was necessary, and yet another said that investment of tax payer money would depend on the country

[5] Respondents could provide multiple answers to this question.

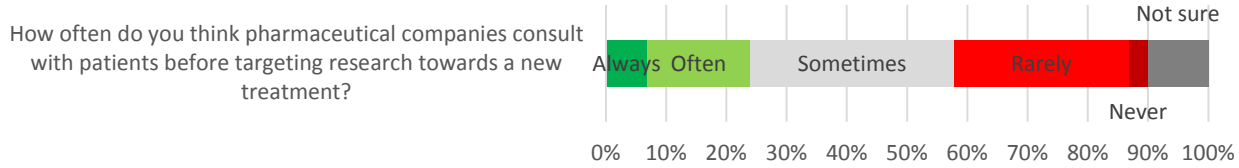
context. Other respondents suggested money should be spent on developing therapies for rare conditions, on investigations into how existing drugs could be used for new conditions, and on educating healthcare professionals about their disease areas. A comprehensive list of comments can be found in Appendix 2: Supplement to questionnaire.

Table 3.16 Preferences on health related public spending.

How would you prefer taxpayer money to be spent on the disease area(s) of your organisation? Select one option	
By supporting the condition outside the health system, for example by providing support to stay at work, home modifications, help with daily activities etc.	34 (21%)
By investing in prevention, for example through life style modification or screening programmes	34 (21%)
By investing in research for new pharmaceuticals	31 (19%)
By increasing funding for existing pharmaceuticals in the health system to make the pharmaceuticals more available to patients	31(19%)
Not sure	16 (10%)
Other	13 (8%)

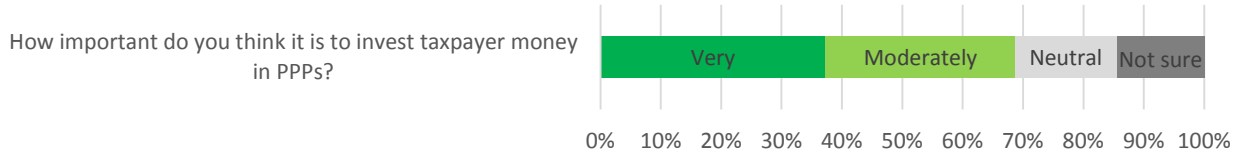
When asked how often respondents thought pharmaceutical companies consulted with patients before targeting research towards a new treatment, more than one third (54 of 159, 34%) thought patients were sometimes consulted, while a similar proportion (46 of 159, 29%) suggested patients were rarely consulted. Only 17% (27 of 159) thought that consultations took place often and less than 10% answered "always" (Figure 3.2).

Figure 3.2 Perception of pharmaceutical companies' patient consultations.



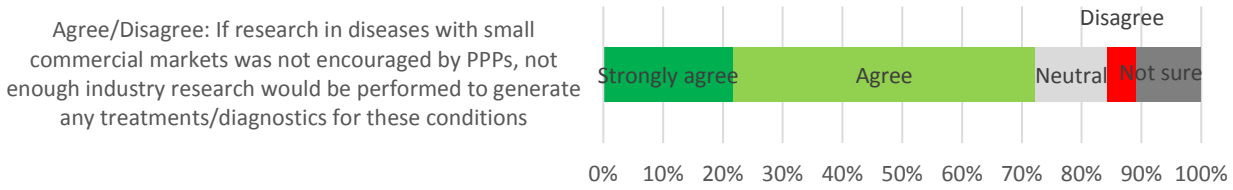
Only respondents who were familiar with PPP's before the survey were asked to answer the following eight optional questions, giving a pool of 97 potential respondents. A majority (57 of 83, 68%) thought it was very or moderately important to invest taxpayer money in PPP's, while none of the respondents found this to be "not at all important" (Figure 3.3).

Figure 3.3 Perception of investment priorities 1.



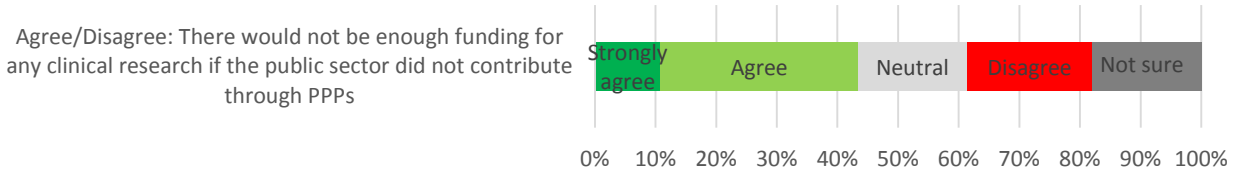
Similarly, a majority of 73% (60 of 83) agreed or strongly agreed that not enough funds would be available for research into conditions with only a small commercial market if not supported by PPP's, while 5% (4 of 83) disagreed (Figure 3.4).

Figure 3.4 Perception of investment priorities 2.



Respondents were more split in the statement about whether there would be enough funding for any clinical research if the public sector did not contribute through PPPs: while a total of 44% (36 of 83) agreed or strongly agreed, 36% (30 of 83) of the respondents were either neutral or not sure, and the remaining 20% (17 of 83) disagreed with the statement (Figure 3.5).

Figure 3.5 Perception of investment priorities 3.

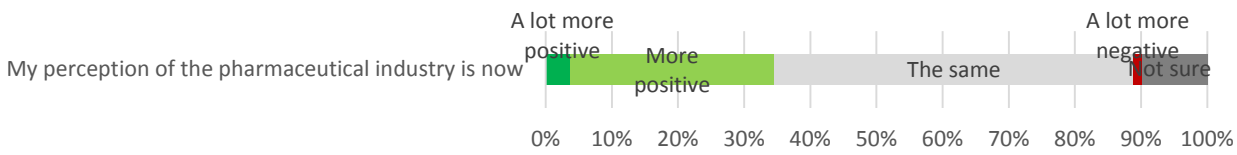


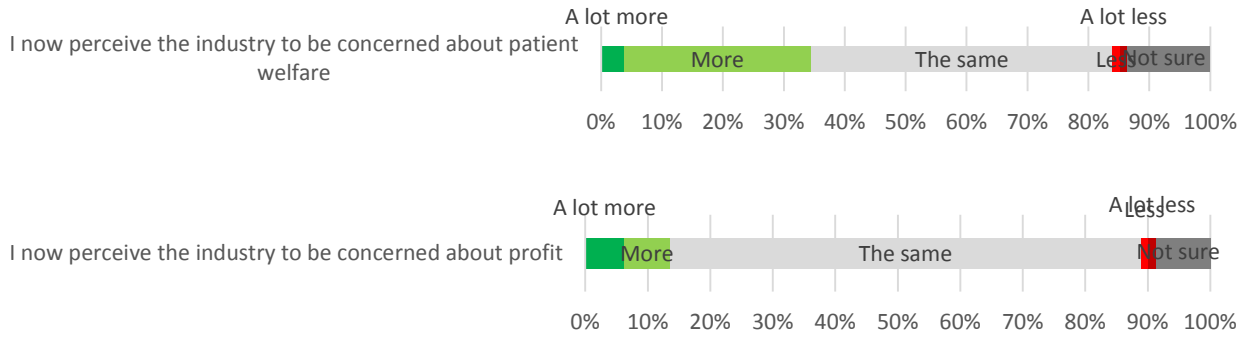
3.10. Perception of the pharmaceutical industry

When asked whether their perception of the pharmaceutical industry had changed knowing the industry was engaged in arrangements such as PPP's (Figure 3.6), the majority (44 of 81, 54%) of respondents stated their perception had not changed, while a third (28 of 81, 35%) had a more positive perception. A single respondent (1 of 81, 1%) stated their perception was now a lot worse.

Along similar lines, 49% (40 of 81) said they did not perceive the industry to be any more or less concerned about patient welfare than before engaging in PPP's, and 35% (28 of 81) now perceived the industry to be more or a lot more concerned with patient welfare. The perception of the industry profit motive however remained largely unchanged when considering PPP's, with a large majority (61 of 81, 75%) not perceiving any change, and a few (11 of 81, 13%) considering the industry to be more concerned about profit.

Figure 3.6 Perceptions of the industry as partner in PPP.

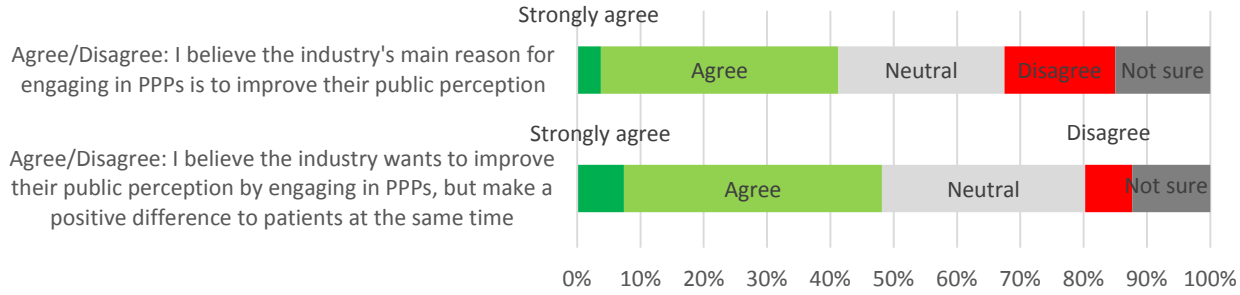




Finally, respondents were asked whether they thought the underlying motivation for the industry's engagement in PPPs was to improve their public perception (Figure 3.7), to which 42% (33 of 80) agreed, while 18% (14 of 80) disagreed, though a large proportion (33 of 80, 41%) either were neutral or not sure.

Similar but slightly more favourable answers were given when respondents were asked if the industry in addition wished to make a positive difference to patients, with 48% (39 of 81) stating agreement, while 7% (6 of 81) disagreed and 44% (36 of 81) were neutral or not sure.

Figure 3.7 Perception of the industries motivation.



3.11. Suggestions from respondents

In the last question respondents were invited to provide comments on ways to improve patient involvement in PPP's such as IMI. A thematic analysis of the comments was conducted. Table 3.17 shows an overview of four emerging themes, the general comment and advice and case references for each theme, as well as the number of respondents touching upon the theme.

Five respondents said that direct contact should be made to improve the communication and partnership among patient organisations and IMI. On a related topic, the information on participation must be clear and accurate, and most importantly, it must be made easily available. Five respondents pointed out issues with information production and dissemination. Third, patient organisations must be given the education and training they need to be able to participate successfully. The need for patient advocate training was emphasised by five respondents. Three respondents voiced the need for a centralised portal with information about on-going research, participation as well as contact information for patient organisations and industry for mutual use.

Two respondents advocated the need for earlier involvement of patients. One respondent emphasised the need for cross-disease groups when involving patients as some disease areas tended to be over represented. Different areas of participation were brought up: one respondent said

patient organisations should be involved in the procurement of pharmaceuticals, while another respondent held patients should be involved in priority setting of research. A comprehensive list of comments can be found in Appendix 2: Supplement to questionnaire.

Table 3.17 Data display: Suggestions for improving patient involvement.

Theme	Comment/Advice	Case reference	# respondents
Direct contact	IMI should contact patient organisations directly, and be present at arenas where patient organisations operate	• "IMI could make a phone call and have a chat with us", • "Systematically contact European umbrella organisations concerned by the disease area of a project to seek patient involvement, advice, and a meaningful partnership." • "Although IDF Europe has expressed an interest in being involved in IMI projects, we have never been contacted and do not know of any diabetes stakeholder such as IDF Europe that has been." • "(…)attending conferences where the patient organisations will be present such as BSPAR"	5
Better information	Information about patient involvement must be clear and easy to access.	• "Patients as well as non-patients, before getting involved in anything, need to be properly informed (…)". • "Improve access to information", • "Information dissemination in a clear and easily understood newsletter / website etc., would make communication easier", "by increase the information and communication about the disease"	5
Patient advocates training	Patients must be given adequate education and training to become successful contributors and partners in pharmaceutical R&D.	• " (…)develop education schemes for patients for participating in research, and create tools for efficient participation", • "Offering training for patients and organizing networks of Patient Research Partners", • "training courses for patient advocacy (…)"	5
Centralised portal	A central portal or platform should be established to facilitate the dissemination of information and ease communication between patients, industry and researchers.	• "Have a central portal that patient organisations can promote to their members and via websites so that patients can see what research is currently going on that they can engage with", • "Patient lists and Company lists should be made and be available to both parties", • "We need some investments to really have a platform to couple researchers, clinicians, patient, funding agencies and industry."	3

4. Analysis

Statistical methods were used to analyse the data and address the objectives set out for the study. The chi-squared test was employed to investigate whether a relationship could be found between two categorical variables, Mann-Whitney's U test was used to test for underlying differences in distributions of ordinal variables between two subgroups, and Spearman Rank Correlation (rho) was used to test association between two ordinal variables. The data was prepared for analysis and the questions of interest were recoded into discrete or ordinal categories to accommodate the statistical tests. An overview of the statistical methods is given in Table 4.9. The results for each test are given in the four sections referred to in the "Objective addressed" column.

4.1. Awareness of IMI

In order to assess the awareness and reasons for awareness (or lack of awareness) of IMI among respondents, several statistical tests were conducted. In particular, respondents working in an organisation at the EU/ European vs. national level, respondents' knowledge about the EU and respondents' knowledge about EFPIA were hypothesised to exhibit some correlation with knowledge about IMI.

As one would expect, a statistically significant correlation was found between having heard about IMI and being familiar with any of the IMI projects (chi-square, $p < 0.001$). Further, a statistically significant relationship was found between knowing what EFPIA was and having heard about IMI (chi-square, $p<0.001$).

Second, a statistically significant correlation was found between having heard about IMI and representing an EU level patient organisation, such that 60% of EU-level and only 34% of national level respondents were aware of IMI (chi-square, $p = 0.005$).

Similarly, a weaker but statistically significant relationship was found between respondents correctly identifying the function of the European Commission and having heard about IMI, with 44% of those aware of IMI correctly identifying the EC vs. 28% among those unaware of IMI (chi-square, $p = 0.043$). Again, this shows a positive relationship between knowing about IMI and having knowledge about the EU.

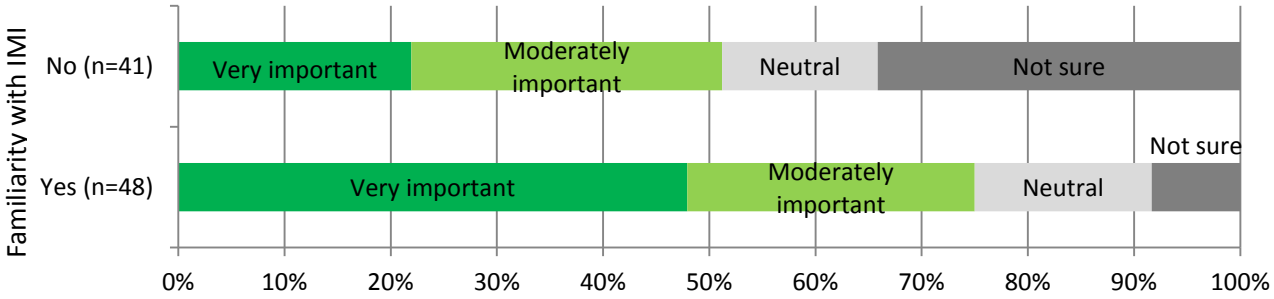
4.2. Perception of value of IMI

Respondents who were familiar with IMI were asked to rate first how important they thought IMI was in driving research on innovative medicines in general and subsequently to rate how relevant IMI's work was in relation to their own organisation. There was a strong association between these answers, such that respondents who thought IMI's work was important in general also thought it was relevant for their organisation (Spearman rank correlation, $p=0.0003$). In turn, respondents who thought IMI's work was more relevant to their organisation were also more likely to be interested in collaborating with IMI (Spearman rank correlation, $p<0.0001$).

More broadly, investing public money in PPPs was rated as important by the majority (68%) of respondents and there was a tendency for respondents who were familiar with IMI to attribute higher importance to investment in PPPs, though this was not statistically significant (Mann-Whitney U, $p=0.1855$) (Figure 4.1).

Figure 4.1 Opinion on investment priorities 1.

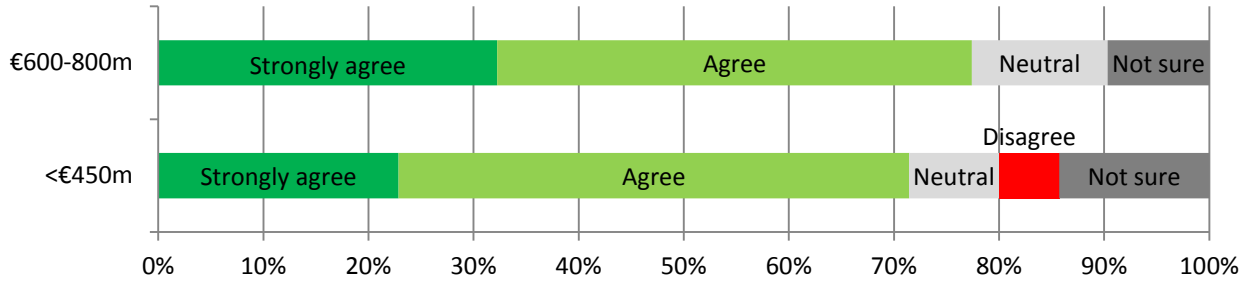
How important do you think it is to invest taxpayer money in PPP's?



While 73% of all respondents thought PPP's were necessary to generate investment in conditions with small commercial markets, there was no substantial or significant difference between those who were familiar with IMI and those who were not (Mann-Whitney U, p=0.4510). On the other hand, respondents who correctly estimated the cost of developing a new drug at €600-800 million tended to agree more that PPP's were necessary to stimulate R&D for conditions with small commercial markets, though this was not statistically significant (Mann-Whitney U, p=0.4588) (Figure 4.2).

Figure 4.2 Opinion on investment priorities 2.

Agree/Disagree: If research in diseases with small commercial markets was not encouraged by PPP's, not enough industry research would be performed to generate any treatments/diagnostics for these conditions

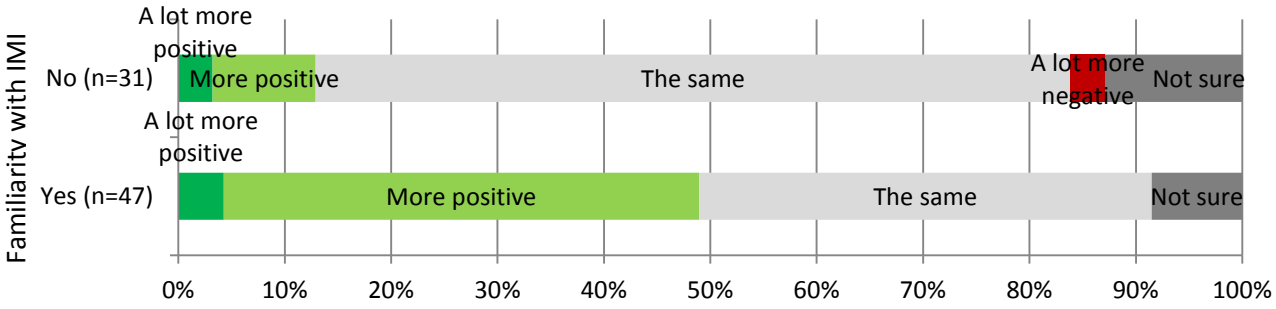


4.3. Perception of the pharmaceutical industry and PPP

The questionnaire results indicated that a small proportion of respondents had become more positive towards the pharmaceutical industry following PPP engagement. There was no difference in the opinion of respondents from EU-level and national organisations (Mann-Whitney U, p=0.96) in this regard, but there was a significant difference between those who were familiar with IMI and those who were not, with knowledge of IMI being associated with a more positive view of the industry after PPP involvement (Mann-Whitney U, p=0.0015) as shown in Figure 4.3.

Figure 4.3 Perception of the industry as partner of PPP 1.

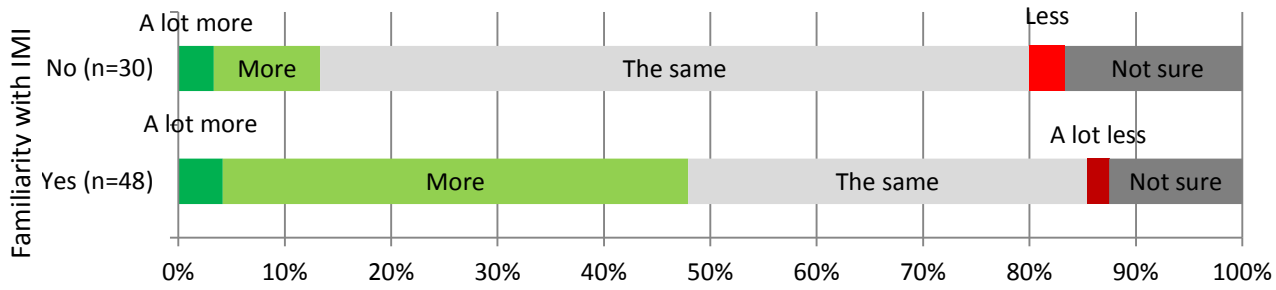
Agree/Disagree: My perception of the pharmaceutical industry is now



The perception of the industry in terms of patient welfare was also largely unchanged following PPP engagement in the sample as a whole, however also in this case knowledge of IMI was associated with a more positive view of the industry, with significantly more respondents answering the industry was more concerned about patient welfare (Mann-Whitney U, p=0.0041) (Figure 4.4). Concerning the profit motive, there was no significant difference between respondents who were aware of IMI or not, nor between respondents representing EU-level or national organisations

Figure 4.4 Perception of the industry as partner of PPP 2.

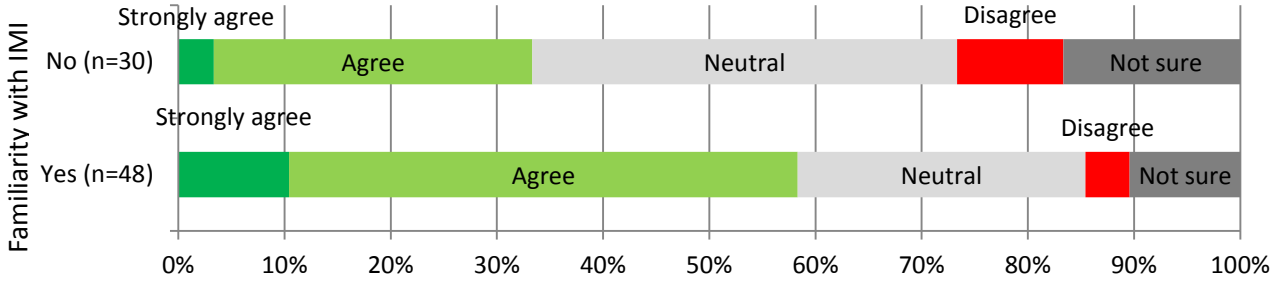
I now perceive the industry to be concerned about patient welfare



There was a tendency among respondents to agree that the main reason for engaging in PPP's was to improve the public perception of the industry. This tendency was qualitatively more pronounced in those who were familiar with IMI, at borderline statistical significance (Mann-Whitney U, p=0.0733).This pattern was more pronounced when the statement combined improving public perception and making a difference to patients at the same time. In this case, those who were familiar with IMI agreed significantly more than those not familiar with IMI (Mann-Whitney U, p=0.0337) (Figure 4.5).

Figure 4.5 Perception of the industry as partner of PPP 3.

I believe the industry wants to improve their public perception by engaging in PPPs, but make a positive difference to patients at the same time

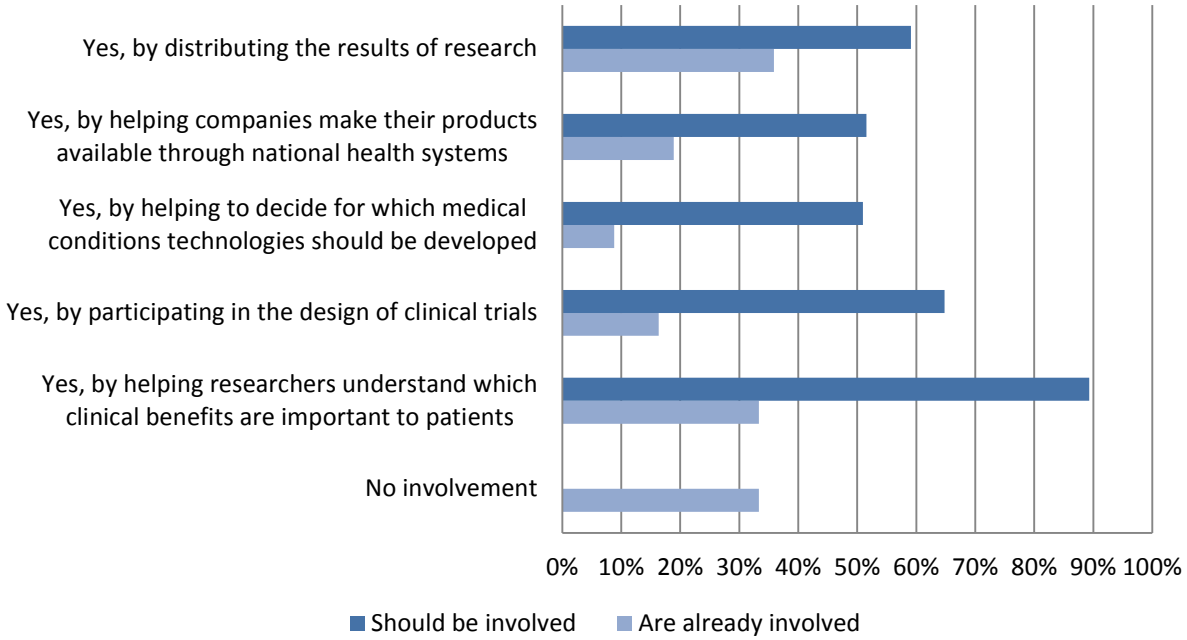


There was also a tendency among respondents to find PPP's valuable in terms of research investment, believing PPP's allowed for research in field that otherwise would get less attention.

4.4. Involvement in IMI

Comparing respondents' own experience of involvement activities with those activities they would like to take part in revealed that while almost all participants felt patient organisations should play a stronger role in helping researchers understand the clinical benefits important to patients, only one third declared that their organisation had participated in this activity. Similarly, while two thirds expressed the need for patient organisation participation in the designing of clinical trials, only one third of the respondents' organisations had done so (Figure 4.6).

Figure 4.6 Mode of involvement wanted and experienced.



4.5. In-depth interviews

Five key figures from patient organisations were interviewed, four female and one male. One informant was engaged in a patient organisation initiative at EU level, two informants in patient organisations at national level (Italy and United Kingdom), and two informants in patient organisation initiatives at both European and national level (Sweden and Israel). A variety of roles within patient organisations were covered: international affairs, external affairs, consultant, volunteer, board member and vice president. Each informant was posed six questions relating to their involvement in clinical research, whether they thought public money should be spent on public private partnerships, whether they thought patients and patient organisations should participate in clinical research and development, what they thought were the main barriers for successful involvement were, and what could be done to overcome these barriers.

Four themes were identified in the data reduction process, each of which relates to different steps in the process of patient participation. Table 4.8 displays the themes and presents the supportive data, underscoring key words from which the coding emerged. Figure 4.7 gives a graphical representation of the process, showing the themes as they appear in the process.

The interview guide can be found in Appendix 3: Interview guide and the interview transcripts can be found in Appendix 4: Interview transcripts.

4.5.1. Change

Three informants (60%) emphasised that things were changing in patient participation, and as was pointed out by one of the informants, in terms of the doctor-patient relationship. “In the last two years the relationship between doctors and patients has changed”. Represented in Figure 4.7 as the atmosphere around the process of patient participation, these changes can be seen as shaping all other attributes, working in favour of the patient. Concrete activities, such as social media and lobbying were pointed out as new ways for patients to make themselves heard. Voicing these changes suggests that patients are positive that patient participation is changing for the better, making it more successful in times to come.

4.5.2. Patient knowledge

All informants (100%) agreed patients were in possession of valuable information that would contribute to clinical research. Being able to provide real life perspectives, feedback and advice were seen as crucial throughout the process. “It is normal to study the consumer when you make a product, what do consumers want and need?”, one informant pointed out when asked whether and how patients should be involved in clinical research. Informants also said giving feedback should be a constant process throughout the lifecycle of medical products rather than a onetime activity. “Also, when a therapy is out on the market we can offer feedback”, one informant said. These attributes are presented as arrows bridging patients and patient participation in Figure 4.7.

4.5.3. Knowledge and information gap

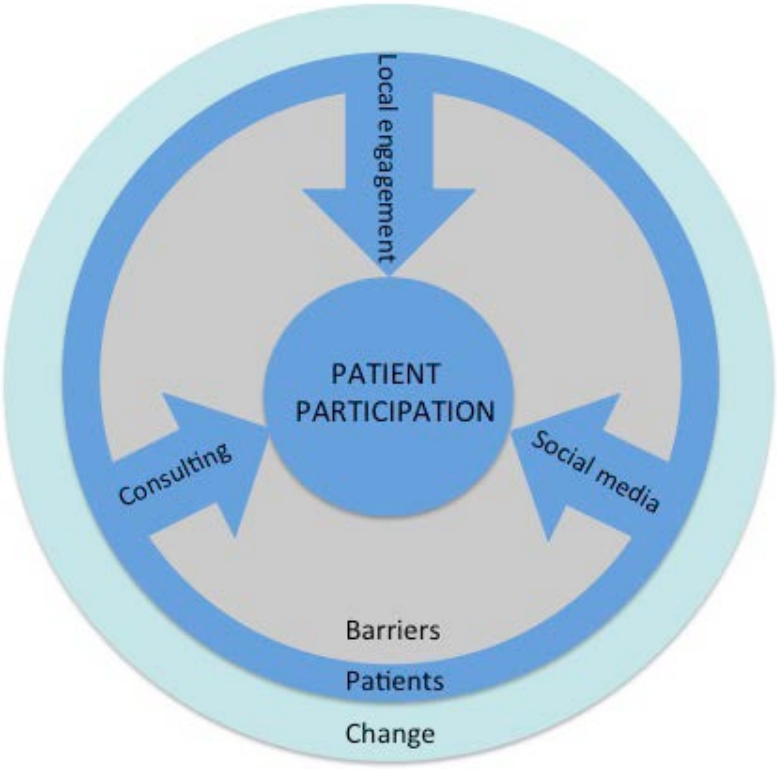
Lacking knowledge or information was seen to be a barrier for patient involvement in two ways. Two informants (40%) pointed out that patients could not contribute to the scientific or technical aspects, but that there were other important tasks to fill as well. One informant claimed that research environments often were “too much medical speak”, making patients feel excluded. To overcome these barriers it was suggested more resources were put into training patients and patient representatives to better face the challenges as experts.

The other way in which lack of knowledge or information was seen as a barrier related to patients not knowing how to participate or with what they could contribute. As one informant put it, "(..) they don't recognise the value of their own knowledge". It was also stated that information on how to participate was not easily available. One informant raised the issue of participating in clinical trials, stating that "there's no central portal for seeing what trials are available and how to join".

4.5.4. Local and grassroots focus

Two informants (40%) emphasised the importance of approaching and engaging patient organisations at the grassroot level, claiming that information must be given as close to the patient as possible to be effective. For IMI to be successful, informants suggested a bottom up local approach was taken, rather than a top down approach through umbrella organisations. "They [umbrella organisations] do not have any direct impact on patients everyday lives and problems they are dealing with", an informant pointed out, stating that there was often a disconnect between the umbrella and the local organisation.

Figure 4.7 Graphical representation of the process of patient participation.



Note: *The graphical representation shows the changing climate in which patient participation is taking place, and patients' means of overcoming certain barriers for successful participation. Different strategies such as social media, consulting and local engagement or bottom up approaches are employed to reach the goal of patient participation.*
Source: The authors

Table 4.1 Display of main themes supported by the data.

Theme		Case reference
The process of patient involvement:	**Change**	We find, though this is getting better, that we are approached at 11th hour for patient participation[N4,Q3]. Doctors can sometimes be "old school", they are the "wise men" and they decide everything for the patients without asking for their preferences (…) But this is changing, and some doctors talk different today. Even in the Faculty of Medicine, students are being taught to speak to patients in a different way. In the last two years the relationship between doctors and patients has changed [N1,Q5]. Difficult to change how people think, this is what they "used to do". This is changing fast because of access to information and the internet [N3,Q5].
Strengths to consider:	**Patient knowledge and perspective**	They came to us in the very beginning so we could help think of the patient and family perspective (…) [N4,Q3]. Doctors are important of course, but patients are the second most important. For example, they can give feedback on secondary (adverse) effects. Also, when a therapy is out on the market, we can offer feedback [N1,Q4]. (…) need to know what their [patients'] needs are and to have constant feedback [N2,Q3]. (…) they [patients] should also be given the opportunity to advise when the (clinical) trials are being formed. Maybe they can't contribute scientifically, but there are other things that are important for the trial [N3,Q4]. Patients can share views on what difference research will make in their lives – or perhaps help to modify the research question slightly to make it more valuable. It's about getting the real life perspective, not performing research for research sake [N4,Q4].
Barriers:	**Knowledge and information gap**	In terms of scientific capabilities they have less than researchers [N6,Q4]. Many do not know how they can participate and they don't recognise (the value of) their own knowledge [N3,Q5]. How user-friendly is the language? Many patients with experience from being involved in research say there is too much jargon and medical speak, making them feel somewhat inadequate and outside their area of expertise. It can get a little highbrow and technical, and they don't feel included. When patients are part of steering committees or panels reviewing funding applications, we need to offer them training to be that expert patient [N5,Q5]. For participating in clinical trials, there's no central portal for seeing what trials are available and how to join [N4,Q5].
Future considerations:	**Local and grassroots focus**	IMI would be working with umbrella organisations – good because they are professionally organised, funded, they have resources and aims. But there is a disconnect between patient organisations at grass root level and the umbrella organisations which are more political, they do not have any direct impact on patients everyday lives and problems they are dealing with [N2,Q6]. But this [information on involving patients] must not only be done at a global level, it also needs to be done locally. A questionnaire to patients is one thing, but if you want them to be involved, the information must be much closer to them [N3,Q6].

Note: N= informant number as marked in the transcripts in Appendix 4: Interview transcripts.
Q=question number as in Appendix 3: Interview guide.

Figure 4.2 Overview of statistical methods used for the analysis.

Objective addressed	Hypothesis	Independent variable	Coding of Independent variable	Dependent variable	Coding of dependent variable	Statistical test	Stata command
Awareness of IMI	There is a positive (/negative) relationship between being familiar (/not familiar) with the IMI and representing (/not representing) an organisation that operates at EU level	Q1: In which country/countries does your organisation operate?	EU/ European level:1, Non EU/European level:0	Q7: Have you heard about IMI?	Yes:1, No:2	Chi-square test	tab q1coded q7coded, chi2
Awareness of IMI	There is a positive (/negative) relationship between being familiar (/not familiar) with IMI and knowing (/not knowing) what the EC is	Q15: Which of the following statements about the European Commission is the most appropriate?	Right answer:1, Wrong answers:0	Q7: Have you heard about IMI?	Yes:1, No:2	Chi-square test	tab q15coded q7coded, chi2
Awareness of IMI	There is a positive (/negative) relationship between being familiar (/not being familiar) with IMI and knowing (/not knowing) what EFPIA is	Q17: Would you describe EFPIA as (select the most appropriate option)	Right answer:1, Wrong answers:0	Q7: Have you heard about IMI?	Yes:1, No:2	Chi-square test	tab q17coded q7coded, chi2
Awareness of IMI	There is a positive (/negative) relationship between being familiar (/not being familiar) with IMI and knowing (/not knowing) what IMI is	Q8: Based on your current knowledge of IMI, would you describe IMI as	Right answer:1, Wrong answers:0	Q7: Have you heard about IMI?	Yes:1, No:2	Chi-square test	tab q8coded q7coded, chi2
Awareness of IMI	There is a positive (/negative) relationship between being familiar with the IMI (/not being familiar) and having heard about one or more of the listed IMI projects	Q14:Please indicate which (if any) of IMI's projects you have heard of	Having heard of one or more:1, Having heard of none:0	Q7: Have you heard about IMI?	Yes:1, No:2	Chi-square test	tab q14coded q7coded, chi2
Perception of value of IMI	Does local/EU-level organisational status affect whether IMI is important in driving research?	Q1: In which country/countries does your organisation operate?	EU: 1, national: 0	Q10: How important do you think IMI is in encouraging and driving research on the development of innovative medicines in general?	1: Very important.. 5: Not at all important	Mann-Whitney U	ranksum q10_a, by(q1coded)

Perception of value of IMI	Is there an association between perception of IMI general importance and importance of IMI to own organisation?	Q11: How relevant do you think research undertaken by IMI is for the patients and the families of patients in your organisation?	Very relevant: 1 .. Not at all relevant: 5	Q10: How important do you think IMI is in encouraging and driving research on the development of innovative medicines in general?	1: Very important.. 5: Not at all important	Spearman rank	spearman q10_a q11_a if (q7 != 2 & q10_a < 6 & q11_a < 6)
Perception of value of IMI	Do respondents who think IMI's work is relevant to their organisations want to collaborate with IMI?	Q11: How relevant do you think research undertaken by IMI is for the patients and the families of patients in your organisation?	Very relevant: 1 .. Not at all relevant: 5	Q12: How interested do you think your organisation would be in participating in IMI's research projects or networking groups that are relevant for your organisation?	Very interested: 1 .. Not at all interested: 5	Spearman rank	spearman q11_a q12_a if (q7 != 2 & q11_a < 6 & q12_a < 6)
Perception of pharma industry and PPP's	Being aware of IMI may have an influence on how the pharmaceutical industry is viewed	Q28: My perception of the pharmaceutical industry is now	A lot more positive: 1 … A lot more negative: 5	Q7: Have you heard about IMI?	Yes:1, No:2	Mann-Whitney U	ranksum q28_a if (q5 != 2 & q28_a < 6), by(q7coded)
Perception of pharma industry and PPP's	Does knowledge of IMI affect perception of whether industry is concerned with patient welfare?	Q29: I now perceive the industry to be concerned about patient welfare	A lot more: 1 … A lot less: 5	Q7: Have you heard about IMI?	Yes:1, No:2	Mann-Whitney U	ranksum q29_a if (q5 != 2 & q29_a < 6), by(q7coded)
Perception of pharma industry and PPP's	Does knowledge of IMI change perception of profit motive?	Q30: I now perceive the industry to be concerned about profit	A lot more: 1 … A lot less: 5	Q7: Have you heard about IMI?	Yes:1, No:2	Mann-Whitney U	ranksum q30_a if (q5 != 2 & q30_a < 6), by(q7coded)
Perception of pharma industry and PPP's	Does knowledge of IMI affect whether PPP's are mainly for improving public perception?	Q31: Agree/Disagree: I believe the industry's main reason for engaging in PPPs is to improve their public perception	Strongly agree: 1 … Strongly disagree: 5	Q7: Have you heard about IMI?	Yes:1, No:2	Mann-Whitney U	ranksum q31_a if (q5 != 2 & q31_a < 6), by(q7coded)
Perception of pharma industry and PPP's	Does IMI familiarity associate with believing PPP's are for public perception AND making a difference to patients?	Q32: Agree/Disagree: I believe the industry wants to improve their public perception by engaging in PPPs, but make a positive difference to	Strongly agree: 1 … Strongly disagree: 5	Q7: Have you heard about IMI?	Yes:1, No:2	Mann-Whitney U	ranksum q32_a if (q5 != 2 & q32_a < 6), by(q7coded)

		patients at the same time					
Perception of pharma industry and PPP's	Does knowledge of IMI result in higher support of investing tax money in PPP?	Q25: How important do you think it is to invest taxpayer money in PPPs?	Very important: 1 .. Not at all important: 4	Q7: Have you heard about the Innovative Medicnes Initiative (IMI)?	Yes: 1, No: 0	Mann-Whitney U	ranksum q25_a if (q25_a < 5), by(q7coded)
Perception of pharma industry and PPP's	Does knowledge of IMI affect attitude to whether PPPs are necessary to generate investment in conditions with small markets?	Q26: Agree/Disagree: If research in diseases with small commercial markets was not encouraged by PPPs, not enough industry research would be performed to generate any treatments/diagnostics for these conditions	Strongly agree: 1 .. Strongly disagree: 5	Q7: Have you heard about the Innovative Medicnes Initiative (IMI)?	Yes: 1, No: 0	Mann-Whitney U	ranksum q26_a if (q26_a < 6), by(q7coded)
Perception of pharma industry and PPP's	Does correct knowledge of pharma drug development cost have an influence on whether PPPs are considered necessary for R&D for drugs with small markets?	Q26: Agree/Disagree: If research in diseases with small commercial markets was not encouraged by PPPs, not enough industry research would be performed to generate any treatments/diagnostics for these conditions	Strongly agree: 1 .. Strongly disagree: 5	Q18: What do you think is the approximate average cost of developing and marketing a new pharmaceutical product?	€600-800m: 1 <€450m: 0	Mann-Whitney U	ranksum q26_a if (q26_a < 6), by(q18correct)
Perception of pharma industry and PPP's	Respondents are more positive	N/A	N/A	N/A	N/A	Descriptive	N/A
Involvement in IMI	Respondents want to participate and some know how	N/A	N/A	N/A	N/A	Descriptive	N/A

5. Conclusions and implications for IMI

Based on the interpretation of the data, several observations may be drawn from the results that overall compare well with the tentative observations from the results of the pilot study (Table 5.1).

Table 5.1 Comparing observations from the pilot study and the large-scale roll out.

Observations from pilot study	Matching observation in large-scale roll out?
Information on R&D relevant to patient organisations is generally sourced from conferences and from within patient organisation networks	*Partly matching.* Information on R&D relevant to patient organisations is generally sourced from conferences and from clinicians and healthcare professionals
EUPATI appears to be the most well-known entity within IMI	*Matching.* EUPATI appears to be the most well-known entity within IMI
Respondents generally feel the work of IMI is important in driving research, and is relevant to patients and families	*Matching.* Respondents generally feel the work of IMI is important in driving research, and is relevant to patients and families
Patient organisations are interested in becoming involved in the work of IMI, in particular in relation to patient training and giving input to the research agenda	*Matching*. Patient organisations are interested in becoming involved in the work of IMI, in particular in relation to patient training and giving input to the research agenda
Patient organisations would like patients to be more strongly involved in helping researchers understand clinical benefit, in the design of trials and in deciding which medical technologies should be prioritised	*Partly matching.* Patient organisations would like patients to be more strongly involved in helping researchers understand clinical benefit, in the design of trials and in dissemination of research
The majority of respondents had not previously participated in any pharmaceutical related research; and the perception of patient input from clinicians and scientists was perceived as a significant barrier	*Partly matching.* Only one third had not previously participated in pharmaceutical related research. However, the perception of patient input from clinicians and scientists was perceived as a significant barrier, together with the claim that patients were not given the opportunity to participate
Respondents feel PPP are a worthwhile way to spend public money	*Matching.* Respondents feel PPP are a worthwhile way to spend public money
Respondents feel more positive about the pharmaceutical industry knowing that they engage in PPP's such as IMI	*Partly matching.* Some respondents feel more positive about the pharmaceutical industry knowing that they engage in PPP's such as IMI, while the majority of respondents have the same perception as before

Further to these observations, the thematic analysis of data collected through parts of the questionnaire inviting respondents to freely express themselves, as well as the analysis of the interview sessions and the quantitative results from the questionnaire allow for some conclusions to be drawn and the emergence of a number of implications and recommendations for IMI's future work on patient involvement.

5.1. Awareness of IMI

39% of the respondents were already familiar with IMI, and positive relationship were found between being familiar with IMI, working in an organisation at EU or European level, having knowledge about the EU and being familiar with pharmaceutical industry actors such as EFPIA. While the most common cause of familiarity with IMI was through conferences, meetings and news, only very few respondents were aware of their organisations being contacted directly by IMI. In practice, this may suggest, that while reaching out to umbrella organisations has been somewhat successful, grassroots organisations are less likely to have heard about IMI.

Recommendation: In order to gain awareness among patient organisations at national and grassroots level, IMI must aim to provide information in both a harmonised and highly contextualised manner, and approach patient organisations directly and at the local level.

5.2. Perceptions of PPP's

While respondents seem to agree PPP's present a valuable way of financing clinical research, there is little evidence to support a substantial change in perception of the pharmaceutical industry. This suggests there may be other aspects that influence patient organisations' perception of the industry, other than financial and partnership arrangements.

Recommendation: If insights into the perception of the pharmaceutical industry are to be investigated, other areas should be explored such as corporate social responsibility, reliability and integrity in order to provide a more complete picture.

5.3. Involvement in research

Patient organisations want to play a bigger role in clinical research, most importantly by advising researchers on the clinical benefits for patients, by participating in the design of clinical trials and in the dissemination of research. However, few have actually participated in these activities, and the barriers for entering the process of participation must be addressed. Lack of information, lack of training, as well as the notion of not being taken seriously, feeling left out or invited only at the last minute are all issues that patient organisations perceive as barriers to successful involvement. Patients must be given adequate education and training if their involvement is to be of any substance and meaning. Furthermore, recruiting patients and patient organisations for involvement is more effective when done at the local level or grassroots level and at an early stage in the process. While patient organisations are in principle interested in participating, they expect the initiative to be taken by IMI. This study suggests that there is unexploited potential for patient involvement.

Recommendation: IMI should aim at better involving patients by focusing on overcoming the barriers. IMI should try to understand patient organisations using a bottom up approach, focusing on grassroots organisations in different geographical contexts. It should also aim to approach organisations in a more direct manner, and facilitate educational and training for those wanting to participate. Finally, IMI should aim to encourage research environments involving patient organisations to be aware of the "medical talk" and discourse used in partnership with patient organisations that might be problematic.

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Appendices

Appendix 1: Questionnaire

This questionnaire aims to collect views on patient involvement in pharmaceutical research. Responses are 100% anonymous. The study is carried out by the Medical Technology Research Group at London School of Economics.

1. *In which country/countries does your organisation operate?*

2. *In which disease area(s) does your organisation work?*

3. *What is your role within the organisation:*
   - ☐ Member
   - ☐ Policy Director
   - ☐ Board Member
   - ☐ Chief Executive
   - ☐ Advisor
   - ☐ Other (please specify):_____

4. *What are your most important sources of information about research and development of interest to your organisation? Select all that apply*
   - ☐ Newsletter, website or colleagues within my own organisation
   - ☐ Newsletters, websites or colleagues from other organisations
   - ☐ Scientific literature
   - ☐ Industry and industry-related newsletters or websites
   - ☐ Clinicians/healthcare professionals
   - ☐ Newspapers or television
   - ☐ Social media (Facebook, Twitter, blogs etc.)
   - ☐ Conferences and other professional gatherings
   - ☐ Specialised bulletins
   - ☐ Other (please specify):_____

5. *Have you heard of the term "public-private partnership" (PPP)?*
   - ☐ Yes
   - ☐ No
   - ☐ Not sure

6. *Can you think of and name any PPPs?*
   - ☐ I have not heard of the term PPP
   - ☐ Yes (please specify):_____
   - ☐ No
   - ☐ Not sure

7. *Have you heard about the Innovative Medicines Initiative (IMI)?*
   - ☐ Yes
   - ☐ No
   - ☐ Not sure

   If you answered No to question 7, please move to question 14.

8. *Based on your current knowledge of IMI, would you describe IMI as:*

- ☐ A private network for North American pharmaceutical companies to share the risk of developing innovative medicines
- ☐ A global partnership of charitable organisations with an interest in health
- ☐ A partnership between the European Union and European pharmaceutical companies to encourage development of innovative medicines
- ☐ A Geneva-based organisation that coordinates public-private partnerships between national governments and the pharmaceutical industry
- ☐ Not sure

9. *How would you best explain why you have heard about IMI?*
   - ☐ My organisation has collaborated with IMI
   - ☐ My organisation has been contacted by IMI
   - ☐ I know of other patient organisations that have collaborated with IMI
   - ☐ I am familiar with IMI more generally through conferences, meetings or news
   - ☐ Other (please specify): _____
   - ☐ Not sure

10. *How important do you think IMI is in encouraging and driving research on the development of innovative medicines in general?*

Very important	Somewhat important	Neutral	Not that important	Not at all important	Not sure
☐	☐	☐	☐	☐	☐

11. *How relevant do you think research undertaken by IMI is for the patients and the families of patients in your organisation?*

Very relevant	Moderately relevant	Neutral	Not that relevant	Not at all relevant	Not sure
☐	☐	☐	☐	☐	☐

12. *How interested do you think your organisation would be in participating in IMI's research projects or networking groups that are relevant for your organisation?*

Very interested	Moderately interested	Neutral	Not that interested	Not at all interested	Not sure
☐	☐	☐	☐	☐	☐

13. *Which type of involvement do you think is likely to be valuable for your organisation if offered to participate in IMI's activities? Select all that apply*
    - ☐ Being directly involved with the companies carrying out research
    - ☐ Being able to give input to IMI's research agenda
    - ☐ Helping to decide which medical areas IMI should focus on
    - ☐ Being involved in projects related to the testing and monitoring of aspects related to the safety and efficacy of pharmaceuticals and diagnostics
    - ☐ Being involved in IMI's education and training projects to enhance patients' understanding of clinical research and development
    - ☐ Other:_____

14. *Please indicate which (if any) of IMI's projects you are familiar with/have heard of (Select all that apply)*

☐ ABIRISK	☐ EMIF	☐ MARCAR	☐ PRO-active

☐ BioVacSafe	☐ EMTRAIN	☐ MIP-DILI	☐ PROTECT
☐ BTCure	☐ eTOX	☐ NEWMEDS	☐ Quic-Concept
☐ CHEM21	☐ eTRIKS	☐ Onco Track	☐ RAPP-ID
☐ COMBACTE	☐ EU-AIMS	☐ Open PHACTS	☐ SAFE-T
☐ COMPACT	☐ Eu2P	☐ ORBITO	☐ SafeSciMET
☐ DDMoRe	☐ EUPATI	☐ Pharma-Cog	☐ STEMBANCC
☐ DIRECT	☐ EUROPAIN	☐ Pharmatrain	☐ SUMMIT
☐ EHR4CR	☐ IMIDIA	☐ Predect	☐ TRANSLOCATION
☐ ELF	☐ K4DD	☐ PreDiCT-TB	☐ U-BIOPRED

15. *Which of the following statements about the European Commission (EC) is the most appropriate?*
    - ☐ The EC works like a national parliament and votes on European legislation
    - ☐ The EC is an assembly of the European heads of state/government responsible for defining the general political direction and priorities of the EU
    - ☐ The EC works like a national government, with departments running various aspects of the EU
    - ☐ Not sure

16. *Does your organisation have any interaction with the EU institutions (the EU Parliament, the EU Commissioner the European Council)? Select all that apply*
    - ☐ My organisation participates in research funded by the EU institutions
    - ☐ My organisation attends conferences organised by the EU institutions on a regular basis
    - ☐ Lobbying
    - ☐ Permanent representation/office in Brussels
    - ☐ Other (please specify):_____
    - ☐ No interaction currently
    - ☐ Not sure

17. *The European Federation of Pharmaceutical Industries and Associations (EFPIA) is a partner of IMI. Would you describe EFPIA as (select the most appropriate option)*
    - ☐ A public-private partnership to increase collaboration within the European pharmaceutical industry
    - ☐ A private industry association representing national pharmaceutical industry associations in Europe
    - ☐ A private research organisation which pools resources from European pharmaceutical companies
    - ☐ Not sure

18. *What do you think is the approximate average cost of developing and marketing a new pharmaceutical product?*
    - ☐ €600-800 million
    - ☐ €300-450 million
    - ☐ Approx. €200million
    - ☐ Less than €80million
    - ☐ Not sure

19. *Who do you think contributes the most money to global R&D on pharmaceuticals?*
    - ☐ The pharmaceutical industry
    - ☐ National and supranational governments
    - ☐ Non-Governmental Organisations, foundations and charities
    - ☐ Approximately equal contribution from governments and the pharmaceutical industry
    - ☐ Not sure

20. *Do you think patients or patient organisations should play a stronger role in pharmaceutical R&D? Select all that apply*
    - ☐ Yes, by helping researchers understand which clinical benefits are important to patients
    - ☐ Yes, by participating in the design of clinical trials
    - ☐ Yes, by helping to decide for which medical conditions technologies should be developed
    - ☐ Yes, by helping companies make their products available through national health systems
    - ☐ Yes, by distributing the results of research
    - ☐ Yes, other (please specify):_____
    - ☐ No
    - ☐ Not sure

21. *Has your organisation participated in any pharmaceutical R&D related activities? Select all that apply*
    - ☐ Yes, by helping researchers understand which clinical benefits are important to patients
    - ☐ Yes, by participating in the design of clinical trials
    - ☐ Yes, by helping to decide for which medical conditions technologies should be developed
    - ☐ Yes, by helping companies make their products available through national health systems
    - ☐ Yes, by distributing the results of research
    - ☐ Other (please specify): _____
    - ☐ No
    - ☐ Not sure

22. *What do you think are the main challenges for successful involvement of patient and patient organisations in R&D? Please select all that apply*
    - ☐ Patient's knowledge is perceived as being less important than knowledge from clinicians
    - ☐ Patient's knowledge is perceived as being less important than knowledge from scientists
    - ☐ It is difficult for a small group of patients to represent the opinions of all patients with the same medical condition
    - ☐ Patients don't know what research is being carried out
    - ☐ Patients are not sure how to approach researches to become involved
    - ☐ Patients are not given the opportunity to become involved by those undertaking research
    - ☐ Other (please specify): _____
    - ☐ Not sure

23. *How would you prefer taxpayer money to be spent on the disease area(s) of your organisation?*
    - ☐ By investing in research for new pharmaceuticals
    - ☐ By increasing funding for existing pharmaceuticals in the health system to make the pharmaceuticals more available to patients
    - ☐ By supporting your condition outside the health system, for example by providing support to stay at work, home modifications, help with daily activities etc.
    - ☐ By investing in prevention, for example through life style modification or screening programmes
    - ☐ Other:_____
    - ☐ Not sure

24. *How often do you think pharmaceutical companies consult with patients before targeting research towards a new treatment?*

Always	Often	Sometimes	Rarely	Never	Not sure
☐	☐	☐	☐	☐	☐

If you were familiar with Public Private Partnerships (PPP) before this survey, please continue from the next question. If you were not familiar with PPPs, please go to the last question (33).

25. *How important do you think it is to invest taxpayer money in PPPs?*

Very important	Moderately important	Neutral	Not at all important	Not sure
☐	☐	☐	☐	☐

26. *Agree/Disagree: If research in diseases with small commercial markets was not encouraged by PPPs, not enough industry research would be performed to generate any treatments/diagnostics for these conditions*

Strongly agree	Agree	Neutral	Disagree	Strongly disagree	Not sure
☐	☐	☐	☐	☐	☐

27. *Agree/Disagree: There would not be enough funding for any clinical research if the public sector did not contribute through PPPs*

Strongly agree	Agree	Neutral	Disagree	Strongly disagree	Not sure
☐	☐	☐	☐	☐	☐

Please consider whether the emergence of PPPs has changed the way you think about the pharmaceutical industry:

28. *My perception of the pharmaceutical industry is now*

A lot more positive	More positive	The same	More negative	A lot more negative	Not sure
☐	☐	☐	☐	☐	☐

29. *I now perceive the industry to be concerned about patient welfare*

A lot more	More	The same	Less	A lot less	Not sure
☐	☐	☐	☐	☐	☐

30. *I now perceive the industry to be concerned about profit*

A lot more	More	The same	Less	A lot less	Not sure
☐	☐	☐	☐	☐	☐

31. *Agree/Disagree: I believe the industry's main reason for engaging in PPPs is to improve their public perception*

Strongly agree	Agree	Neutral	Disagree	Strongly disagree	Not sure
☐	☐	☐	☐	☐	☐

32. *Agree/Disagree: I believe the industry wants to improve their public perception by engaging in PPPs, but make a positive difference to patients at the same time*

Strongly agree	Agree	Neutral	Disagree	Strongly disagree	Not sure
☐	☐	☐	☐	☐	☐

33. *Can you think of and list other ways to involve patients and patient organisations in PPPs such as IMI?*

Thank you for your participation!

Appendix 2: Supplement to questionnaire

The below tables are copies of tables presented in the results section including comprehensive lists of comments provided by respondents in the field "Other" for questions providing this alternative. The comments have been edited mildly to ensure clarity and correct spelling.

Table 2.3: Can you think of and name any PPPs?	
Hospitals and the IMI	IMI
IMI as the most important	The IMI initiative as well as EUPATI
IMI	IMI
ec.europa.eu/internal_market/publicprocurement	IMI, Global Fund (TB, Malaria, AIDS), GAVI
Hospital de La Ribera, Spain	PPP between the pharmaceutical industry and government
EUPATI	Drugs for Neglected Diseases Initiative
IMI	AutoCure, BTCURE,
Institut du Cerveau et de la Moelle (Paris)	PFI hospital building
IMI	French Telethon and INSERM
IMI, London Underground (2003 -2010), London Organising Committee for the 2012 Olympic Games (a private company but guaranteed with public money)	Hospital building programme
IMI	EUPATI
IMI and others	FP7 projects
At the local level, partnership for some health centres in Catalunya	PatientPartner Europe under 7th Framework
IMI and others	Medicines Information Project in the UK
Regional hospital in France	NHS
Construction of government projects e.g hospitals	Capital building projects
IMI	Autocure, BTcure, Combine, IMI
EUPATI - European Patients' Academy on Therapeutic Innovation	EUPATI, IMI
I do not know exactly	local authority with an NGO
Top life sciences partnerships in Netherlands	In other field than medicine, yes
IMI	IMI
IMI	Pharma 6G
School building	In my town but also school building and social housing

Table 2.9: Does your organisation have any interaction with the EU institutions (the EU Parliament, the EU Commission, the European Council)? Select all that apply	
No interaction currently	62
My organisation attends conferences organised by the EU institutions on a regular basis	46
Lobbying	40
My organisation participates in research funded by the EU institutions	30
Other	19
Permanent representation/office in Brussels	14
Not sure	12

Other:

- I am also a board member of Pain Alliance Europe with offices in Brussels
- Punctual collaboration
- Members of The European Federation of Allergy and Airways Diseases Patients Associations (EFA)
- Secretariat for the MEPs Against Cancer in the European Parliament
- Meetings with the European Commissioners
- Only participate in some meetings
- Via pan European Cancer Patient Coalitions (ECPC)
- Member of Federation of European Scleroderma Associations (FESCA) promoting cross-European research related to Scleroderma
- We are members of European Pain Association which has had interactions with the EU
- Interaction in some issues related to Multiple Sclerosis
- Grant administrator for a European Cooperation in Science and Technology project
- As a National Member organisation of European Haemophilia Consortium we interact with EU institutions
- No direct interaction. More effective to lobby via collaborative groups in similar disease areas
- Through EURORDIS and the European Haemophilia Consortium (EHC)
- We have some contacts with Maltese representatives and ministers in the EU however we have not made use of these contacts for anything specific as yet.
- Respond to consultations
- Indirect relation via the European Federation of Neurological Associations
- Permanent office and lobbying indirectly through the European Haemophilia Consortium, a European umbrella organization

Table 2.13: Do you think patients or patient organisations should play a stronger role in pharmaceutical R&D? Select all that apply	
Yes, by helping researchers understand which clinical benefits are important to patients	142
Yes, by participating in the design of clinical trials	103
Yes, by distributing the results of research	94
Yes, by helping companies make their products available through national health systems	82
Yes, by helping to decide for which medical conditions technologies should be developed	81
Other	9
Not sure	3
No	0

Other:
- Our participation in clinical trial design is very limited and non-technical.
- Partnerships are a useful approach. Some patient organisations are more politically active/effective as pressure groups than others. It would be unfair to leave decisions on developments purely to the 'loudest and biggest' of patient organisations.
- Yes, by being involved in national HTA-processes for innovative therapies - if they are really innovative.
- In distributing the results of research I would assume that they would have access to it which is not always (if ever) the case.
- By funding the research. Research cannot be restricted to the government and industry
- Partnerships
- By helping ensure drugs are marketed at a price that is fair to the industry and to society
- Funding research
- Taking part in clinical trials

Table 2.14: Has your organisation participated in any pharmaceutical R&D related activities?	
Yes, by distributing the results of research	57
No	53
Yes, by helping researchers understand which clinical benefits are important to patients	53
Yes, by helping companies make their products available through national health systems	30
Yes, by participating in the design of clinical trials	26
Not sure	24
Yes, by helping to decide for which medical conditions technologies should be developed	14
Other	13
Other: - Our members have individually. - Some of our members may have. - I am relatively new in the post as CEO - hence the additional 'not sure' response. We intend to disseminate 'news worthy' results of research in the future. - Our organisation holds two meetings annually for industry members that highlight the importance of involving patients in the development of medicines as well as current and upcoming hurdles. - Not yet but we shall take part in these activities. - I am sure EULAR did - but am not totally clear in which ways. - Not yet. But we are about to. - A local research team (in finding a cure for Parkinson's Disease) has delivered two talks to our members informing us of their results. We are now going to publish their abstracts on our new website and liaise with them to possibly fund their research article publications in the future. - Patient recruitment. - We have been invited now and then to tell about our work, our wishes and needs. That has been a nice experience for us. - Funding research. - Not directly but our members have had involvement in all of the above. - Taking part in clinical trials	

Table 2.15: What do you think are the main challenges for successful involvement of patient and patient organisations in R&D?	
Patient's knowledge is perceived as being less important than knowledge from clinicians	104
Patients are not given the opportunity to become involved by those undertaking research	101
Patient's knowledge is perceived as being less important than knowledge from scientists	97
Patients don't know what research is being carried out	92
Patients are not sure how to approach researches to become involved	89
It is difficult for a small group of patients to represent the opinions of all patients with the same medical condition	67
Other	18
Not sure	6

Other:

- Patients may not be interested in research
- Patient organisations are not given support and training to participate fully in research, and are also approached as an "after thought" or at the 11th hour to find a patient partner.
- Communication difficulties exist and information asymmetry predominates. This makes discourse difficult but not impossible. Co-production of research is therefore very unlikely, although information transfer is possible.
- Where a condition is little known and understood, is complex and where the presentation of the condition/disease is similar to another condition, it can cloud the issue and understanding of the 'main' condition or disease
- Patients should be educated for this
- Training of patient advocates to be able to participate. Funding/financing the time of patient advocates in research. Currently in the industry: Lack of awareness about why patient groups should be involved, what the values and benefits are.
- We get involved in a project/consultation when approached but we do not have the resources to get involved on a regular basis. We get involved in development of clinical guidelines.
- Patients are not offered training to become “Patient Research Partners”
- All answers depend of course on the patient group in question. It is true that the patients' voice is becoming increasingly valued however not enough. Training of patients is very important to ensure their confidence in contributing and being involved with R&D, but there are many success stories out there.
- Funding of patient organisations has been cut down substantially in the Netherlands. It is difficult to maintain a professional organisation.
- It is in general not possible to represent all patients' views. Hence a small group can/should only represent their views and to get a broader view quantitative research is necessary. There is not enough understanding of the issues involved, not enough processes to facilitate this involvement, much needs to be done to make involvement/participation of patients in research the norm. Standard approaches need to be re-written and a whole generation of researchers needs to learn these approaches until this will really be accomplished.
- Scientists and patients need to work together. Patient organisations need to promote research as a full part of the patients' issue and solution.
- If our organisations were approached asking for patient involvement in research I am sure our members would be interested. I have heard of projects where patients were asked to phone in so that an analysis of their voice could be made. When I tried to inquire further I was not successful. So there may be some missed opportunities out there.
- Even when patients or patient organisations are involved, their views are listened to but then not acted on when decisions are made.
- Information is not communication.
- Small patient organisations struggle to obtain funds for research. Patients' condition make it difficult for them to take part in research.
- Usually patient organisations are small organisations, sometimes without any employees. Being involved requires time. Most patient organisations are lacking time to do so.
- Patients' needs are often not perceived as a priority in the research agenda.

Table 2.16: How would you prefer taxpayer money to be spent on the disease area(s) of your organisation? Select one option	
By supporting the condition outside the health system, for example by providing support to stay at work, home modifications, help with daily activities etc.	34
By investing in prevention, for example through life style modification or screening programmes	34
By investing in research for new pharmaceuticals	31
By increasing funding for existing pharmaceuticals in the health system to make the pharmaceuticals more available to patients	31
Not sure	16
Other	13
Other: - By investing in European national patient organisations to support their activities and enable them to become more autonomous and effective at a policy and decision making level. E.g. helping to ensure that the right person gets the right treatment at the right time. - All of these are required. - To investigate and understand the mechanisms and commonalities for connective tissue disease conditions and consider pharmaceutical developments to address underlying malfunctions of the immune system. In addition, specific drug therapies for rare conditions plus continued investigation into how a drug developed for one condition might also benefit patients with another condition. - A mixture of activities: Investing in research for innovative therapies. Investing in current therapies so the healthcare system can get the most value out of the existing therapies (adherence, therapy- and side effect management, dosing, duration, treatment beyond progression, etc.) Investing in studies for additional procedures, such as surgery and radiation. Investing in basic research to find more/better targets and biomarkers for targeted drugs. - Education of healthcare professionals in pain management; making it routine to ask patients about their pain and assess it. - Basic research into the autoimmune disease mechanism. Access to treatment according to the recommended standard of care (clinical and medical). Social reforms to support and assist people with difficulties to have equal opportunities as "normal" people. Much more focus on coping and self-management by provision of education and training programmes. - All of the above to greater and lesser extents. - By investing in research on cheaper therapy (production methods) or gene therapy. - Difficult to say because it depends on the health care system in our country. Romania is not Germany. Investing in new pharmaceuticals of course, but also to actually cure the disease would be our main goal. Then prevention and providing support to patients. - By funding new medications, for example Benlysta. At this time we cannot get it. But I think it should be at least given to children and youngster when helpful for them in getting an education and starting adult life. - All of these are important and it is not possible to prioritise one over another. - By investing in the organisation and human capacity building of patient organisations to become reliable and competent partners for policy makers and industry. - By improving the access to and quality of the disease specialised services.	

Table 2.17: Can you think of and list other ways to involve patients and patient organisations in PPPs such as IMI?
IMI could make a phone call and have a chat with us.
Patients as well as non-patients, before getting involved in anything, need to be properly informed and, in this respect, it is required to have a real strategy which will equally apply and be accessible to all patients in all European countries. Lately, the trend has been to focus on the so-called Western Europe and much less on the EE member states.
Communication and marketing strategy, attending conferences where the patient organisations will be present such as British Society for Paediatric and Adolescent Rheumatology (BSPAR)
I used the ABPI's "Time to Flourish" document as a template for patient engagement. If you want a copy please contact me **simon.davies@teenagecancertrust.org**
In developing projects.
Not sure.
By making their names available to pharmaceutical companies for surveys.
Have a central portal that patient organisations can promote to their members and via websites so that patients can see what research is currently going on that they can engage with.
Yes. Demographically driven demand is outstripping the capacity of governments to fund healthcare. Various policy responses have been developed to mitigate. Patient self-management involving acceptance of responsibility where possible is an area that could be pursued. This would require acceptance from the professionals since there would be an inevitable diminution of their sovereignty. Underpinning that from entry into the basic educational system would be a dedicated teaching programme that focused on wellness, body monitoring and health maintenance. The challenge would be to achieve awareness by school leaving age. This would require a radical programme. Finally we need to think of better ways to die in the social rather than biological sense. These suggestions are for demand reduction programmes rather than stimulating increases in supply (of therapies). As such they may not meet IMI criteria although perhaps industry should reorient itself beyond the confines it places on its remit in response.
Potentially, clinical trials and feedback where this is ethical and feasible - with robust safety controls and open feedback from patients - linked to/monitored by respected experts from the field relevant to the specific rare disease. There would need to be benefits to both the medical institution and to the Patient Organisation.
Disseminate information about IMI, involve patient organisations in national research networks, develop education schemes for patients for participating in research, and create tools for efficient participation.
Promote research in rare diseases. Explain how a chronic disease affects daily life and is not just a theoretical (scientific) issue. Patients' trust and respect should always take precedence over marketing strategies and advertising.
Even though I said I did not know about PPP, I did hear about it at the EULAR conference in Madrid this June. I attended a PARE (People with Arthritis/Rheumatism in Europe EULAR Standing Committe) on patient-partnership and became very interested. This has not yet been done in Finnish autoimmune circles and I am looking for ways to change this, with the help of Lupus Europe.
Patient lists and company lists should be made and made available to both parties, so that patients could become more involved, and perhaps have a chance to gain access to the development of life saving treatments.
Lobbying more among health professionals, GP's and patient organisations. Offering training for patients and organising networks of Patient Research Partners.
More communication with patients and patient organisations.
National grant organisations in Netherlands are really developing patient participation in basic and clinical research at all phases (1 to 4 of clinical research). We need some investments to really have a platform to couple researchers, clinicians, patients, funding agencies and industry. We are underway but not there yet.
Patients should be given opportunities to get informed and gain knowledge.
Generate better working relationships with patient organisations at an earlier stage. Give greater respect and value to the views of patients. Eliminate the "token" patient. Ensure that when patient organisations are involved, their time/effort is valued and funded.
Patients should be involved in priority setting of research items. Translational research has to be encouraged. Patients like to see a cure, the sooner the better. Quality of life has to be enhanced.
Systematically contact European umbrella organisations concerned with the disease area of a project to seek

patient involvement, advice and a meaningful partnership. Although International Diabetes Federation (IDF) Europe has expressed an interest in being involved in IMI projects, we have never been contacted and do not know of any diabetes stakeholder such as IDF Europe that has been.
Improve access to information - in my country mostly doctors have it and it is not shared with others than those they invite to participate. Also, improve feedback - release results of research (both positive and negative). I also think that sometimes the industry to some extent manipulates patients - we had a case many years ago where a company asked a patient organisation for a letter of support for a new medicine, but eventually it turned out that this locally produced medicine did not meet the quality and efficiency of international medicines, however due to the cheaper price we had no choice but to use it. I think this should not be the situation and organisations like IMI should have the authority to supervise such actions by the industry.
Offer project-based funding to small charities with strong patient and caregiver involvement. Too often money is wasted 're-inventing the wheel'. With what is considered small amounts, additional funds could help small organisations to strengthen opportunities for patients to contribute to a generic, collective voice.
Information dissemination in a clear and easily understood newsletter/website etc. would make communication easier. Directly asking for patient involvement when this is required. Families of patients may also be required for research involvement e.g. in trying to investigate further into genetics etc. There is only one research project for Parkinson's Disease in Malta at the moment - so for us it is important to liaise with other countries to be involved as much as possible. Making the contacts is always hard therefore if there was a way of sharing contacts that would be great.
Only by getting a few organisations involved from the beginning – there is a distinct afterthought-type feeling as in "we've got so far, now get us some patients and quick!"
Provide funding opportunities that allow patients to cover their costs in contributing to PPP's, also e.g. compensation of volunteer time that would otherwise be "invested" into the patient organisation and not in the PPP.
To collaborate in the presentation of new health technologies into the National Health Systems. For example, a new technology may be denied acceptance or funding due to risks involved in its utilisation. However, patients often suffer much more without the medication than they would with the possible risk and side effects of taking it. Present arrangements do not allow this leeway in most countries.
Direct contact.
In training courses for patient advocacy involving also families of patients.
For patient organisations to be involved in the procurement process of their product/medication.
Patient organisation in the committee. Questionnaire to the patient organisation on how they think they can be involved. Questionnaire to the pharmaceutical company on how they think patient organisation can be helpful for a new drug.
Feedback from patients on their objectives regarding their disease (i.e. pain control, staying at work etc.)
Ensure the involvement of cross-disease patient groups. Some disease areas can be overrepresented.
By addressing the need for training and recruitment of professionals. By increasing information and communication about the disease, by increasing patients' empowerment, by providing services the public sector is not able to provide etc.

Appendix 3: Interview guide for in-depth semi-structured interviews

Name:

Job/ Role:

Patient organisation:

1. *Are you familiar with IMI and/or any IMI projects?*
2. *Do you think public money should be invested in PPP – and why?*
3. *Are you/your organisation involved in any pharmaceutical/health research?*
4. *Do you think patients/ patient organisations should be involved in research – and why?*
5. *What do you think are the most significant barriers for patients/ patient organisations to become involved in research?*
6. *How do you think IMI could better involve patients and patient organisations in their activities?*

Appendix 4: Interview transcripts

Informant 1: Adriana Carluccio (Italy)

Job/ Position: Responsible for international relations

Patient organisation: Associazione Persone con Malattie Reumatiche (APMAR)

1. Are you familiar with IMI and/or any IMI projects?

Yes, I am a little bit familiar with the IMI because my boss, the president of our organisation, attended a meeting with the IMI this summer in Brussels. So I am a little bit familiar with it from what my boss told me. EULAR (the European League Against Rheumatism) is participating in a project with the IMI, but I can't remember the name of the project.

2. Do you think public money should be invested in PPP – and why?

Yes, as a patient I think that is important. The world is changing fast, and we must invest more money in research. A lot of things are happening in Italy, and we might have to pay for drugs that today are free (to the patient).

3. Are you/your organisation involved in any pharmaceutical/health research?

Yes we are involved in a research, to try to understand if patient are willing to pay for their treatments or not, with different scenarios such cost and efficacy percentage of how much we are willing to pay.

4. Do you think patients/ patient organisations should be involved in research – and why?

Yes, because I think it is better if the patient can help and give feedback. Doctors are important of course, but patients are the second most important. For example, they can give feedback on secondary (adverse) effects. Also, when a therapy is out on the market, we can offer feedback. This is important because patients get money from the government when they can't work. If they get the treatment they can be more productive and pay back through taxes. It is an everyday fight.

5. What do you think are the most significant barriers for patients/ patient organisations to become involved in research?

Doctors can sometimes be "old school", they are the "wise men" and they decide everything for the patients without asking for their preferences. For example, they can decide between a pill and injection without consulting the patient. But this is changing, and some doctors talk different today. Even in the Faculty of Medicine, students are being taught to speak to patients in a different way. In the last two years the relationship between doctors and patients has changed. This is also thanks to patients complaining and use of social media as "the new lobby", which has become very popular. For example, government stopped paying for a treatment for patients with rheumatism, and through social media people gathered from all over the country in Rome to complain. The government had to give in, so it worked.

Another problem is that there are sicknesses of first level and of second level, and only some diseases get attention from media or politicians. These days, diabetes and child obesity get a lot of attention, maybe because the pharmaceutical industry are pushing from the other side.

6. How do you think IMI could better involve patients and patient organisations in their activities?

I think the IMI already are involving patients in their activities. Our president (of the organisation) attended that meeting in Brussels. Also, patient organisations can spread information (about IMI and projects).

Informant 2: Tsveta Schyns (EU level)

Job/ Role and patient organisation: Member of Paediatric Committee, EMA, Independent consultant: EUReMS, Volunteer: ENRAH, EURORDIS

1. Are you familiar with IMI and/or any IMI projects?

Yes. Following several of their projects on Schizophrenia, cognitive area and Alzheimers. Following calls and been to meetings most recently in June. Following EUPATI and other projects dealing with information for clinicians and patients.

2. Do you think public money should be invested in PPP – and why?

It's good to have these initiatives – important to have leadership from the public side, as it is now from what I observe, the industry is initiating projects and deciding the type of research. Not necessarily bad, they can move things and have resources, but academia has been on the back-side until now, the initiative hasn't come from the public side.

3. Are you/your organisation involved in any pharmaceutical/health research?

Not directly. The current project with clinical and rehabilitation centersand patients is co-funded by industry, no participation otherwise.

4. Do you think patients should be involved in research – and why?

Yes absolutely – the question is how. It's important because in the end patients are the users – need to know what their needs are and to have constant feedback. There's also clinical research in which patients are participants. It's normal to study the consumer when you make a product – what do consumers want and need?

The environment has not been supportive for basic researchers to do this –if you focus on a molecular basis in basic research, you try to see the big picture, what's it all about? Basic researchers are not in direct contact with patients to the same extent. They do not necessarily have to deal with patients. For clinical research there is much more to be done to allow patients to be involved, not just as study participants but as partners .

5. What do you think are the most significant barriers for patients to become involved in research?

Difficult to change how people think, this is what they "used to do". This is changing fast because of access to information and the internet. It's not difficult to find out what research is going on and where. It's amatter of somebody facilitating the process. Also the patients have to push –the initiative starts with the patient. Researchers don't know where to find patients – it's more logical that the interest comes from patients. There's increasingly more pressure for things to be transparent and online, making information more widely available.

There's a geographical component – in some areas there are many clinical trials. Barriers exist for people in some countries. It's language barriers, cultural differences, less civil society (fewer patient organisations).

6. Any recommendations for IMI on involving patients?

It's a very good thought to involve patients – but it has to be carefully planned. So many different levels of involvement – is it individual patients, or what kind of patient orgs? IMI would be working with umbrella organisations – good because they are professionally organised, funded, they have resources and aims.

But there is a disconnect between patient organisations at grass root level and the umbrella organisations which are more political, they do not have any direct impact on patients everyday lives and problems they are dealing with. So, how to involve the "other" organisations at the grass roots. It is an area you need to plan well, first to understand and how to work with these organisations, it's so heterogeneous, the ways the organisations are set up and work. Eg. Belgium and Bulgaria don't support charities much, so it's difficult for patient organisations to operate. These cultural differences exist between countries, there are geographical differences. Level of professionalism – it's all volunteer work, depends who is behind the organisation. So the question is: what do you want to achieve? It's also different between disease areas. In rare diseases, there is more individual involvement, there's nobody for us to do the job.

Informant 3: Susanna Lindvall (Sweden, EU level)

Job/ Role: Vice president of EPDA (European Parkinson's Disease Association) and Board member of the Swedish Parkinson's Disease Association.

Patient organisation: EPDA (European Parkinson's Disease Association) and Parkinson Forbundet (Swedish Parkinson's Disease Association)

1. Are you familiar with IMI and/or any IMI projects?

Yes, I have heard about the IMI, but I don't know their specific projects, only that they exist.

2. Do you think public money should be invested in PPP – and why?

Yes, I think they should because I think they (PPPs) have a lot of good initiatives that they can bring to the table. They can contribute with other parts that are important for science.

What parts?

There are different kinds of initiatives that do different things, and I think the IMI is more focused on innovative initiatives, as the name suggests. Such initiatives (PPPs) can maybe better involve various stakeholders.

3. Are you/your organisation involved in any pharmaceutical/health research?

Yes, we partner in three EC projects namely REPLACES – that has just finished and two others NRT, REMPARK and REPLACES –(http://www.epda.eu.com/en/projects/epda-partnered-projects/eu-projects/) Our partnership involvement is dissemination of information about the projects using publications, website and social media

4. Do you think patient/ patient organisations should be involved in research – and why?

Yes, I do think patients should be involved in research because they are the ones participating in the clinical trials. Therefore, they should also be given the opportunity to advise when the (clinical) trials are being formed. Maybe they can't contribute scientifically, but there are other things that are important for the trial. This can benefit the patients because they can get more effective therapy. Also, when the trial ends, their doctor will be more familiar with both the patient and the treatment. Patients will also benefit (from participating in research) and from the satisfaction it gives to help others. Also, they want to move science further. And the most important thing, of course, it contributes to get a more effective treatment.

5. What do you think are the most significant barriers for patient/ patient organisations to become involved in research?

First of all they are not invited to participate. Second, they don't know how to do it (participate). Many do not know how they can participate and they don't recognise (the value of) their own knowledge. For example, to be part of a clinical trial, you cannot just sign up. It is very dependent on which doctor has which study. Some university hospitals allow you to say on their website that you want to participate. But sometimes, this information is really difficult to find. For example, at Karolinska (Stockholm's biggest university hospital) they do trial allowance online, but it is very difficult to find it if you don't know about it. Therefore clear, accurate signposting is essential if patients are to be able to have the opportunity to be more closely involved and contribute to the debate.

6. How do you think IMI could better involve patients and patient organisations in their activities? What is your advice to IMI?

The main thing is more information about how they can participate. But this must not only be done at a global level, it also needs to be done locally. A questionnaire to patients is one thing, but if you want them to be involved, the information must be much closer to them. Same comment as above i.e. therefore clear, accurate signposting is essential if patients are to be able to have the opportunity to be more closely involved and contribute to the debate.

At the EPDA we are developing a leaflet about the benefits of participating in clinical trials. This is being translated in to several languages, and is not disease specific. This will also benefit people from different disease areas. The Swedish pharmaceutical industry association is doing something

similar, but I think it is important that this should be unbiased. I think this (leaflet) can be important in informing people about the benefits of participation.

Informant 4: Clare Jacklin (United Kingdom)

Job: Director of External Affairs, National Rheumatoid Arthritis Society

Patient organisation: covers whole of UK. Focusing on rheumatoid arthritis **–** help line, engage with professionals, stakeholders, advocacy and campaigning (e.g. NICE, BSR, EULAR PARE), groups throughout the UK with peer-to-peer support.

1. Are you familiar with IMI and/or any IMI projects?

Not at all

2. Do you think public money should be invested in PPP – and why?

Yes – without investment in looking at innovative medicines, where else will it come from? It is good to have a counterbalance to purely private investment.

3. Are you/your organisation involved in any pharmaceutical/health research?

We have been approached by wide range of academics doing clinic and social research. We are also involved with INVOLVE, in putting together a panel of expert patients to review funding applications for clinical research, and have hosted many focus groups with academic partners – e.g. on how to recruit for clinical trials and the information needed by patients, and on getting people on steering committees to ensure the patient voice is heard. We find, though this is getting better, that we are often approached at 11th hour for patient participation, i.e. late in planning process. This should really happen in the beginning since patients are the recipients of research. When we are not involved from the beginning, decisions are already made and our involvement is more like a rubber stamp – whereas being involved from the start we could have had more influence and contributed to better outcomes.

We have not been much involved in private industry research, but we are partly funded from private industry. We have been involved in social impact of RA – what does remission mean to patients (this was with industry - Roche). But we more often work with academics, e.g. Guys Hospital where a group of researchers are looking at targeted medicine and running a focus group to understand how patients and their families respond to the notion of people with particular risk factors for RA taking toxic drugs before developing the condition. They came to us in the very beginning so we could help think of the patient and family perspective, and understand what type of questions to be answered when beginning a clinical trial.

4. Do you think patients should be involved in research – and why?

Yes, this is very important – when starting research you always have in mind what you're expecting to find. Some research you think "what was the point of that?" If there is no benefit to an individual, is it worth spending money on that research? Patients can share views on what difference research

will make in their lives – or perhaps help to modify the research question slightly to make it more valuable. It's about getting the real life perspective, not performing research for research sake. Also, patients can help to understand what the common questions and concerns are for patients potentially joining a clinical trial, making recruitment more accessible.

5. What do you think are the most significant barriers for patients to become involved in research?

How do patients know about research in the first place? How user-friendly is the language? Many patients with experience from being involved in research say there is too much jargon and medical speak, making them feel somewhat inadequate and outside their area of expertise. It can get a little highbrow and technical, and they don't feel included. When patients are part of steering committees or panels reviewing funding applications, we need to offer them training to be that expert patient. Currently there is no funding anywhere for delivering that training. For participating in clinical trials, there's no central portal for seeing what trials are available and how to join.

6. How do you think IMI could better involve patients and patient organisations in their activities?

Talking to patient organisations and offering to facilitate some training to get panels of expert patients for each organisation, which they can access easily as needed. And any new research to be shared via central portal on the internet that the general public can access to learn more about engaging in research opportunities.

Informant 5: Amichai Arieli (Israel)

Job/ Role: Retired professor of Animal nutrition

Patient organisation: European Parkinson's Disease Association, Israeli PD Association

1. Are you familiar with IMI and/or any IMI projects?

No.

2. Are you familiar with PPPs?

No

3. Do you think public money should be invested in PPPs?

It makes sense, I don't know exactly the rules, but seems to make sense.

4. Are you/your organisation involved in any pharmaceutical/health research?

The Israeli organisation is trying to build relations with lobby groups in the parliament. EPDA I think has some relationship with the EU Parliament in terms of lobbying.

5. What is the strategy of the Israeli organisation for becoming involved in research?

Some groups of medical professionals want to undertake research so they ask the organisation. We have been approached – we don't have much money, but still we are suggested to give stipends for

new doctors to start research programme about PD in Israel. It is a process for us, but we are really not much engaged in research, we are more involved in helping our members, not in finding a solution for the disease; this needs much more money than we can raise.

6. Do you think patients should be involved in research – and why?

Generally yes – in order to undertake research you need people (as subjects), but how to do it I don't know. In terms of scientific capabilities they have less than researchers, but it is good to involve patients in checking ideas, building the field, maybe not so much in the technical aspects of research but more in general perspective. The Industry interest (profit) may, theoretically at least, vary from patient interest (welfare). Hence patients should be represented in boards dealing with public money usage.

7. What do you think are the most significant barriers for patients to become involved in research?

Sometimes they are afraid of taking risks of a medical intervention, usually when I think about research there are different kinds – let's say some aspects of walking or falling, somebody checks how your walking is through a survey or camera, you don't take much risk. But if you are taking a new medicine, you are not sure how it will affect you, so you are more reluctant.

7. What advice would you give to researchers trying to involve patients?

Make sure that people are not going to be influenced in the wrong way, many people would like to help and find solutions, but you have to ensure that they are not going to be hurt. It's not a question of money or incentives.