



The Role of Patient Advocacy Groups

A Patient/Caregiver Study by Rare Patient Voice

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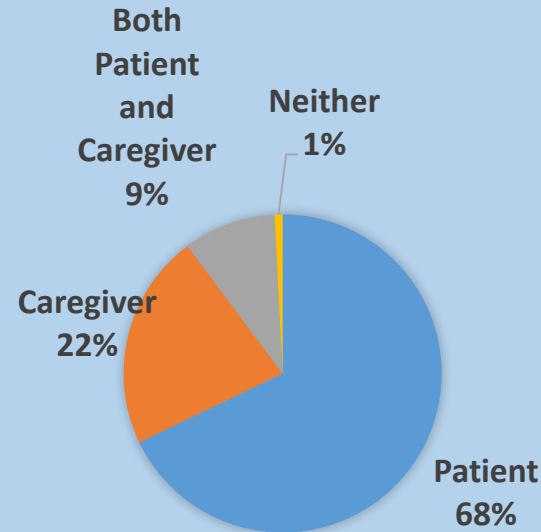
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Market Research Survey among Rare Patient Voice patient/caregiver panel

- N=3455
 - 2344 patients
 - 757 caregivers
 - 326 both patients and caregivers
 - 28 Neither
- Conducted March 30 – April 7, 2017
- US only



150+ Diseases Represented

22Q 11.2 Deletion	Alzheimer's	Behcet's	COPD	Fibromyalgia	Hodgkin's Lymphoma	Lymphedema	Ovarian Cancer	Pulmonary Arterial Hypertension	Syringomyelia
Abdominal Migraine	Anaphylaxis	Bipolar	Crohn's Disease	Friedreich's Ataxia	Huntington's Disease	Mantle Cell Lymphoma	Pancreatic Cancer	Raynaud's Syndrome	Testicular Cancer
Achalasia	Ankylosing Spondylitis	Bladder Cancer	Cushing's Disease	Gastroparesis	Hydrocephalus	Mast Cell Activation Syndrome	Panhypopituitarism	Rheumatoid Arthritis	Tetralogy Of Fallot
Achondroplasia	Anxiety	Breast Cancer	Cystic Fibrosis	Gaucher Disease	Hypoparathyroidism	Melanoma	Paroxysmal Nocturnal Hemoglobinuria	Schizencephaly	Thyroid Cancer
Acromegaly	Aplastic Anemia	Cardiomyopathy	Degenerative Disc Disease	Glutaric Aciduria, Type 1	Hypothyroidism	Meniere's	Parkinson's Disease	Scleroderma	Tourettes
Acute Myelocytic Leukemia	Asthma	Celiac Disease	Diabetes	Guillain Barre Syndrome	Idiopathic Pulmonary Fibrosis	Mesothelioma	Pemphigus Vulgaris	Sickle Cell Disease	Trigeminal Neuralgia
Addison's Disease	Ataxia	Cerebral Palsy	Duchenne Muscular Dystrophy	Hashimoto's Thyroiditis	Immune Thrombocytopenia Purpura	Mitochondrial Disease	Pitt Hopkins Syndrome	Sjogren's Syndrome	Tuberous Sclerosis
ADHD	Atrial Fibrillation	Cervical Cancer	Ehlers Danlos Syndrome	Head And Neck Cancer	Kidney Cancer	MPS	Polycythemia Vera	Sleep Apnea	Ulcerative Colitis
Adult Growth Hormone Deficiency	Autism	Chiari Malformation	Eosinophilic Esophagitis	Hemophilia	Lennox Gastaut Syndrome	Multiple Myeloma	Prader Willy Syndrome	Spina Bifida	Uterine Cancer
Alagille Syndrome	Avascular Necrosis	CIDP	Epilepsy	Hepatitis C	Liver Cancer	Multiple Sclerosis	Primary Immunodeficiency	Spinal Muscular Atrophy	Vitiligo
Alpha-1 Antitrypsin Deficiency	Barth Syndrome	Colon Cancer	Fabry Disease	Hereditary Angioedema	Lung Cancer	Neutropenia	Psoriatic Arthritis	Still's Disease	Von Willebrand Disease
ALS	Beckwith Wiedemann Syndrome	Common Variable Immune Deficiency	Familial Adenomatous Polyposis	HIV	Lupus	Non Small Cell Lung Cancer	PTSD	Stroke	Waldenstroms Macroglobulinemia



800+ Advocacy Groups Represented

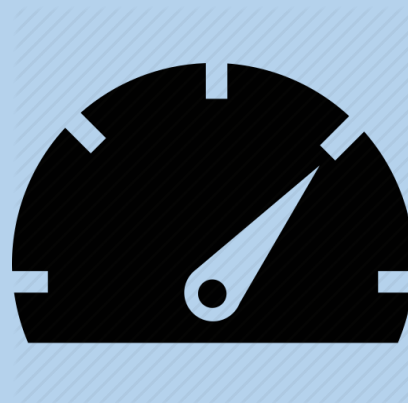
National Multiple Sclerosis Society	International Waldenstrom's Macroglobulinemia Foundation	Susan G. Komen Foundation	International Pemphigus and Pemphigoid Foundation	American Diabetes Association	Arthritis Foundation	American Cancer Society - Cancer Action Network	Rare Patient Voice	Hemophilia of Indiana	The Mastocytosis Society
Cystic Fibrosis Foundation	Multiple Sclerosis Association of America	Shared Solutions	Lupus Foundation of America	Cancer Support Community	Bleeding disorders alliance of illinois	American Heart Association	APDA	inspire.com	ThyCa
Multiple Sclerosis Society	Crohn's and Colitis Foundation of America	Leukemia and Lymphoma Society	National Psoriasis Foundation	Hydrocephalus Association	Fabry Support and Information Group (FSIG)	Cancer support group	Autism Society	Living Beyond Breast Cancer	Triple Negative Breast Cancer Foundation
American Cancer Society	WhatNext	Multiple Sclerosis Alliance	Aplastic Anemia/MDS Intl. Foundation	Metavivor	Food Allergy Research & Education (FARE)	FOD Support Group	Autism Speaks	MPS Society	US Pain Foundation
Huntington Disease Society of America	Epilepsy foundation	ClusterBusters	Dysautonomia International	Michael J. Fox Foundation	Hearing Loss Association of America	Friedreich's Ataxia Research Alliance (FARA)	Bonnie J. Addario Lung Cancer Foundation	MS lunch Club	We Are Rare
Pulmonary Hypertension Association	Lennox Gastaut Syndrome Foundation	Juvenile Diabetes Research Foundation	MS Lifelines	Ms support group	MS One to One	MitoAction	Breast Cancer Coalition of Rochester	Myasthenia Gravis Foundation of America	Wisconsin Bleeding Disorders Network
Immune Deficiency Foundation	Ehlers-Danlos Society	Sjogren's Syndrome Foundation	Muscular Dystrophy Association	National Organization for Rare Disorders (NORD)	National Ataxia Foundation	MS support	Coalition for Hemophilia B	National Tay Sachs and Allied Diseases	Adrenal Insufficiency United
We're Not Drunk We Have MS	MAGIC Foundation	CURED	Pancreatic Cancer Action Network	Sickle Cell Disease Association of America	National Gaucher Foundation	NMD United	Colon Cancer Alliance	New England Hemophilia Association	Aids Foundation Houston
National Hemophilia Foundation	Tuberous Scelrosis Alliance	GBS/CIDP Foundation International	Platelet Disorder Support Association	United Mitochondrial Disease Foundation	PatientsLikeMe	Organic Acidemia Association	Ehlers Danlos National Foundation	Pheo Para Troopers	Alpha1.org
Cure SMA	Hemophilia Federation of America	Multiple Sclerosis Foundation	Spondylitis Association of America	American Partnership for Eosinophilic Disorders (APFED)	Scleroderma Foundation	Pulmonary Fibrosis Foundation	Ehlers-Danlos National Foundation (EDNF)	PSI	AMC



Topics Covered: Perceptions of Advocacy Groups



**Involvement with
Advocacy Groups**



**Advocacy Group
Performance**



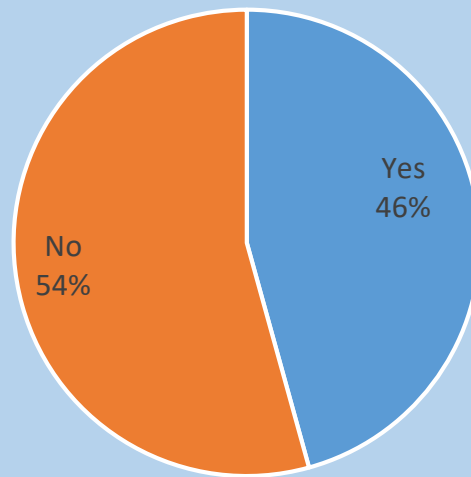
**Partnership with
Biopharmaceuticals**



Less than half of the patients/caregivers are involved with an advocacy group



Are you involved with any advocacy groups that work for you or your loved one's disease/condition(s)?



N=3341



Many don't work with an advocacy groups due to lack of awareness



Why don't you work with an advocacy group? (check all that apply)



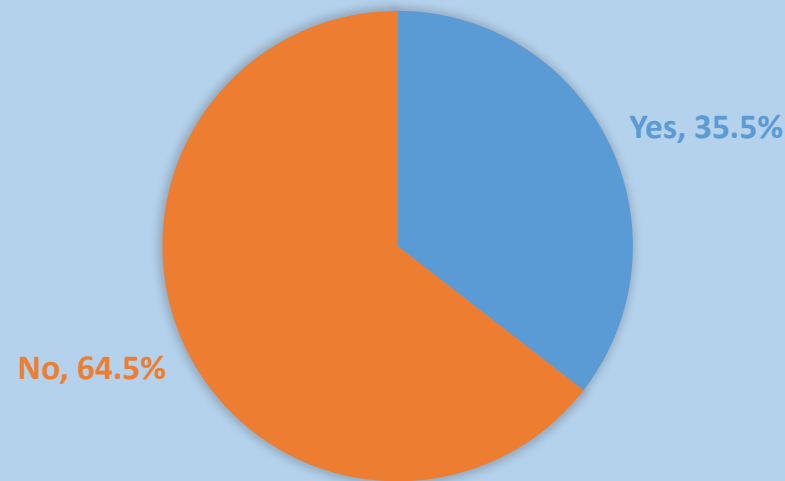
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Among those who don't work with an advocacy group, most aren't aware of one.



Are you aware of an advocacy group or groups that work for you or your loved one's disease/condition(s)?



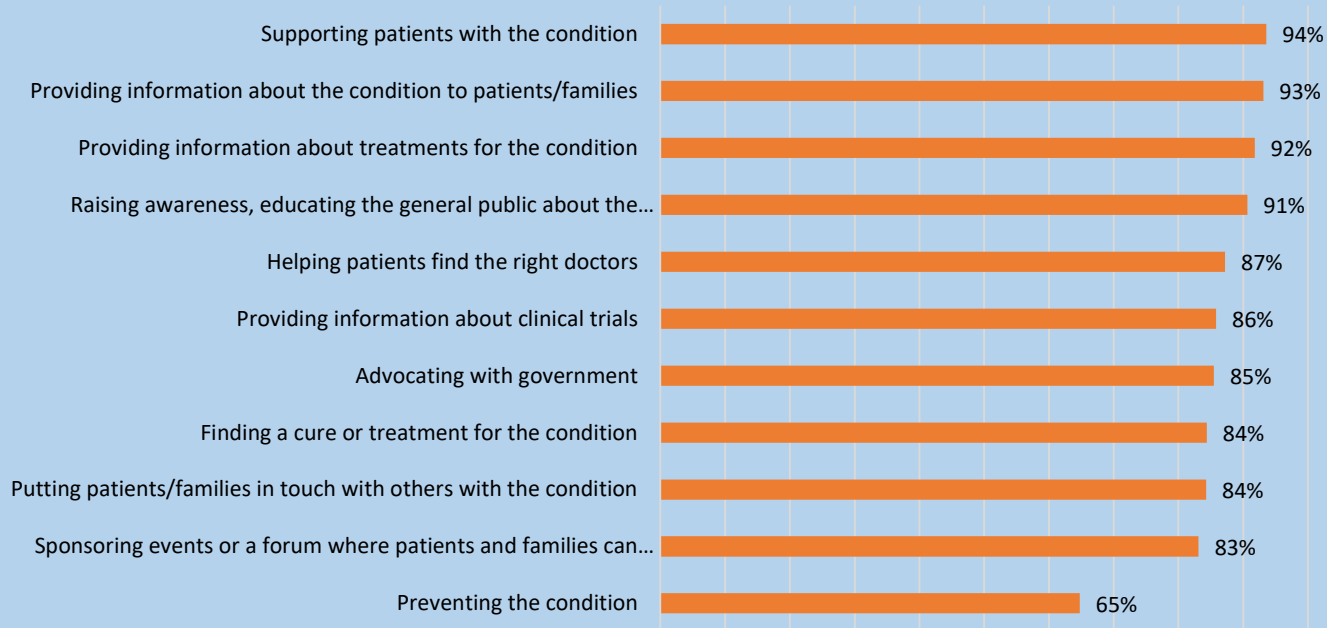
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Many functions of advocacy groups are seen as important, especially supporting patients, providing information and raising awareness.



How important do you feel each of the following advocacy group activities is? Important/Very Important (Top 2 Box)



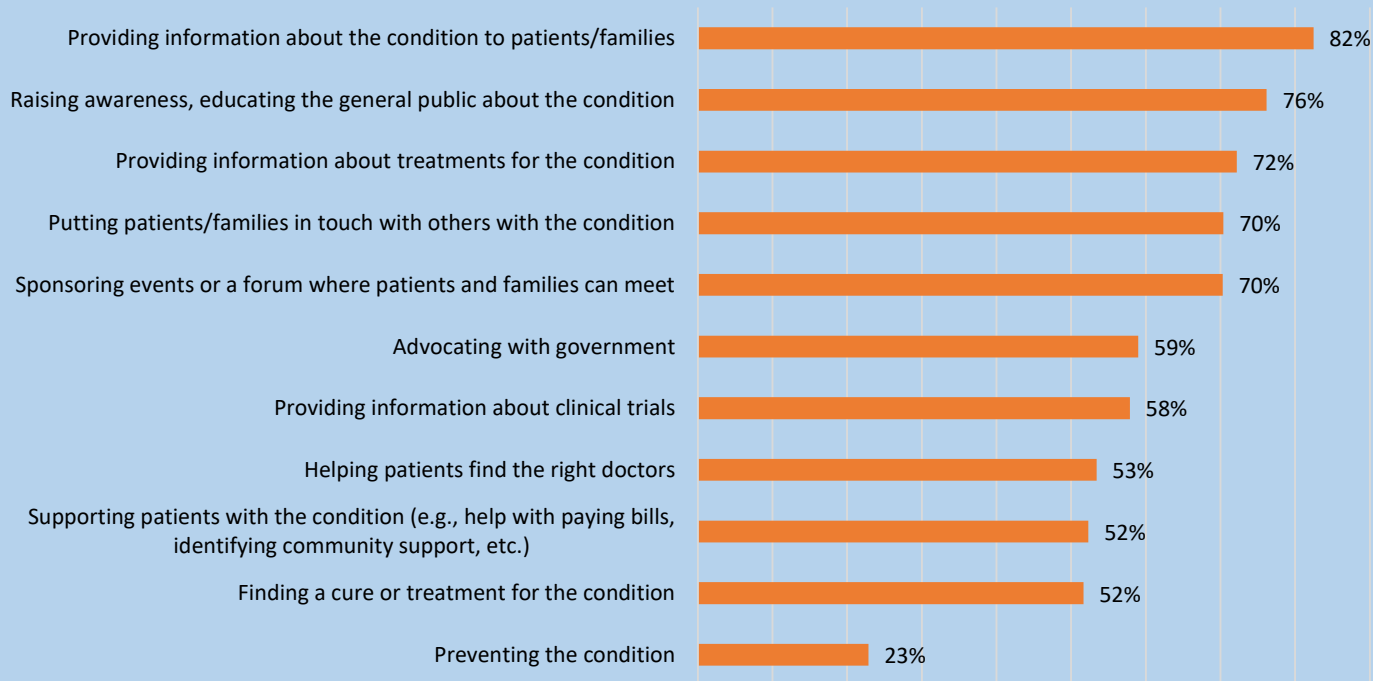
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Advocacy groups are seen as most proficient at providing information and raising awareness.



Thinking of the advocacy group you mentioned, how well does it perform on each of these goals or activities? Well/Very Well (Top 2 Box)



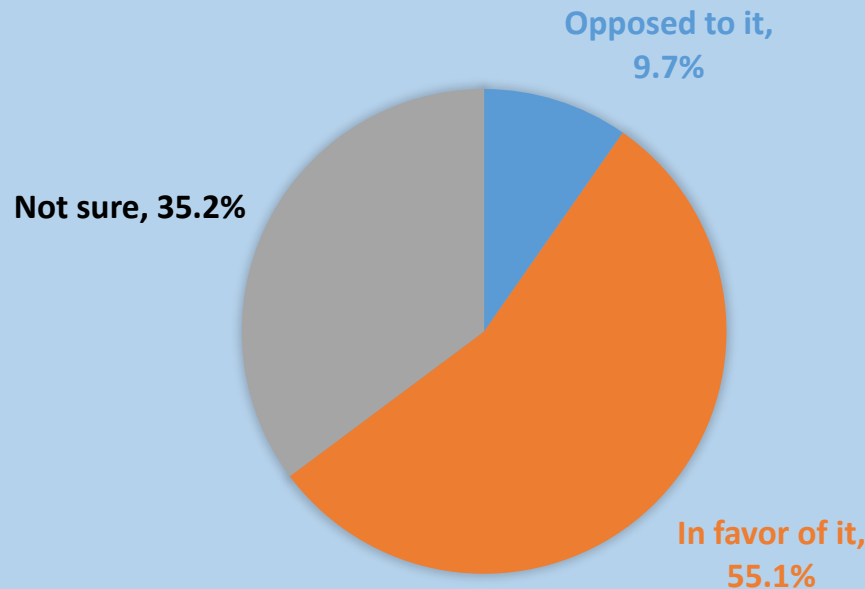
N=1948



A majority of patients/caregivers are in favor of partnerships between advocacy groups and pharmaceutical companies.



Some advocacy groups partner with pharmaceutical or biotechnology companies to develop drugs for medical conditions. Are you in favor of this, or opposed to it?



N=3003



Why are you in favor of these partnerships?



“I am in favor of ANYTHING that will help!” Huntington’s caregiver

“Advocacy groups are more aware of the ups and downs of a patient and can give a better understanding as to what needs to be included in the development of the needed drugs.” Hepatitis C patient

“Any help in research, funding, and treating diseases are expensive and impossible without the help of pharmaceutical companies.” Pemphigus patient

“It might speed up lifesaving treatment.” Turner’s Syndrome caregiver



Why are you opposed to these partnerships?



“Advocacy groups should remain impartial to treatment options.” Juvenile Diabetes caregiver

“I don't believe drugs are always the answer.”
Trisomy X patient

“Advocacy groups should be separate so they are able to provide a true voice for their members.” Hemophilia B patient

“Because the Big Pharma is out to make money...period!”
Multiple Sclerosis patient



Why are you not sure about these partnerships?



“They should be in contact, but they should not be heavily influenced. One is for the patient, the other is for profit.”
Hemophilia A patient

“This can be positive, but it also may feel like the advocacy group is too involved with pharma to really work for what's best for patients.” Common Variable Immune Deficiency patient

“There is a very fine line between partnering with a pharma company and in turn becoming an advocate for the drug product - it's a very tricky situation.” Crohn's patient

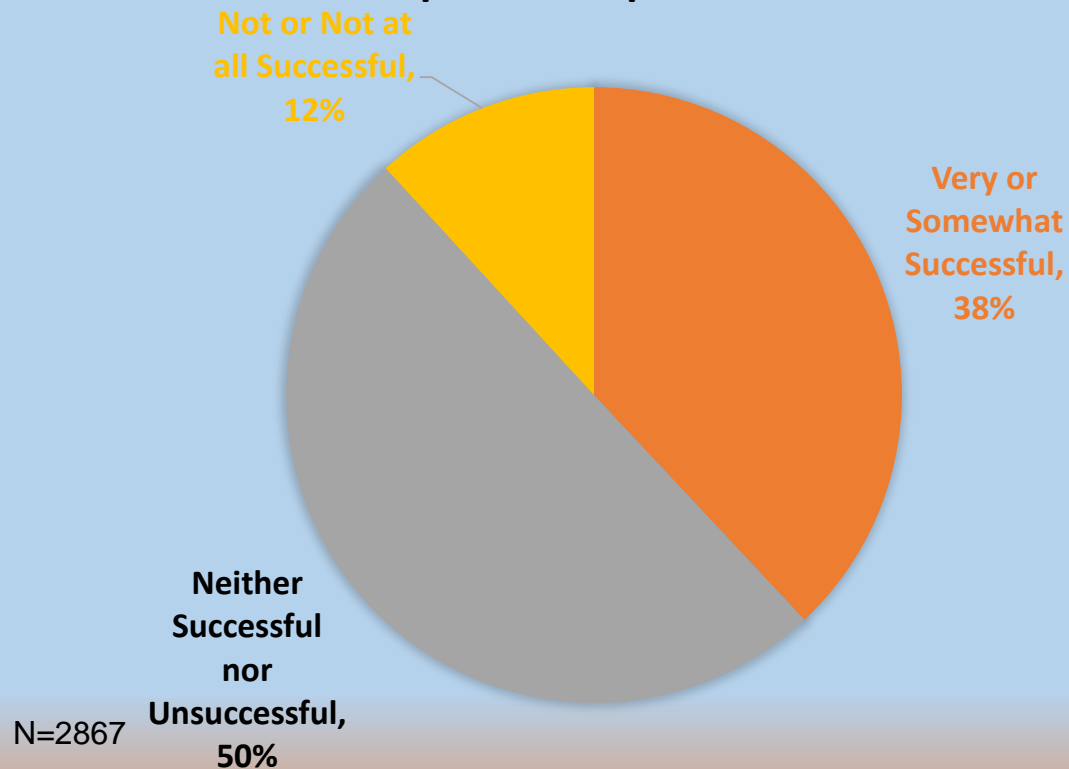
“If it were to help the patients I'm for it but I would not want an advocacy group pushing a specific product.” Von Willebrand Disease patient



Patients/Caregivers have a somewhat positive view of the success of the partnerships.



How successful do you think these partnerships are?



Comments on Partnerships

“At least in the case of the CF Foundation... amazing prototype of how to do it! It's a win-win for everyone!”
Cystic Fibrosis caregiver

“When the scientists meet those living with the disease, it adds a human element and serves to highly motivate those with the brains smart enough to effect a cure.” Spinal Muscular Atrophy caregiver

“Depends on how it's managed. In my observation it is successful when there is an open dialogue and interest between the community and the industry and then within the community itself. The community must be interested and invested in being engaged.” Childhood cancer survivor, massive radiation damage

“It would seem they would be helpful if the advocacy is in it truly for the patients & would be able to bring concerns from patient stand point as well.” Spinal Muscular Atrophy Caregiver

“Probably very successful, however it troubles me that drugs which are found to be effective are then out of financial reach for most people.”
Myotubular myopathy caregiver



Summary

- Lack of awareness of advocacy groups is a key barrier to working with them.
- Many functions of advocacy groups are seen as important, especially supporting patients, providing information and raising awareness.
- Advocacy groups are seen as most proficient at providing information and raising awareness.
- Although a majority of patients/caregivers are in favor of partnerships between advocacy groups and pharmaceutical companies, many are unsure, and the minority who are opposed have strong feelings.
- Some of those supporting partnerships feel that partnerships can enable drug development to take patient perspectives into account, that it is the best way to obtain funding, that it can accelerate the development of treatments, and that anything that might help obtain a cure or improvement should be tried.
- Those opposed fear loss of independence for the advocacy group, a conflict of interest where profit motive takes priority over patient needs, and simply a feeling that drugs may not be the answer.
- Those who are unsure see the benefit of cooperation, but are also concerned about a conflict of interest.



Recommendations

- Increase Awareness
 - Advocacy groups need to increase awareness of their existence and their services to the community they strive to serve.
 - Working more with specialists who diagnose rare conditions, and their office staff, to spread the word might increase awareness as patients are diagnosed and need information.
- Advocacy/Pharmaceutical Partnerships
 - To gain greater support of their members, advocacy groups looking to partner with biopharmaceutical firms should make sure to communicate the benefits of the partnership to patients.
 - They should also take steps to ensure and demonstrate that the advocacy group, while cooperating, will maintain its independence and will make patients' needs its priority over promoting a specific treatment.

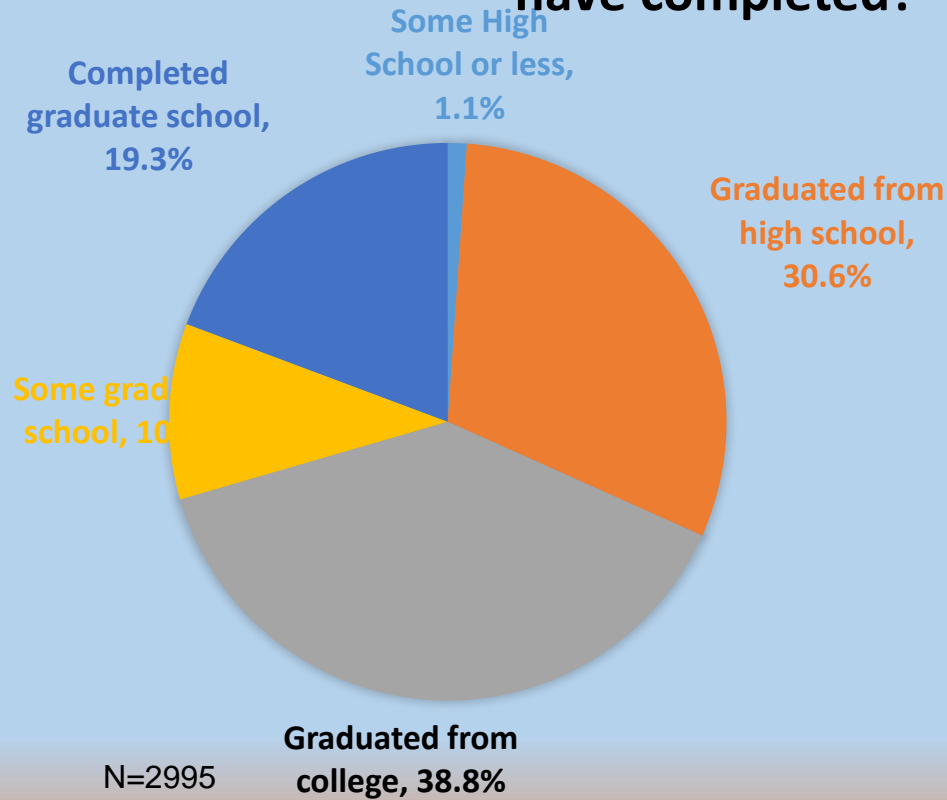


Appendix



Educational Level

What is the highest level of education you have completed?

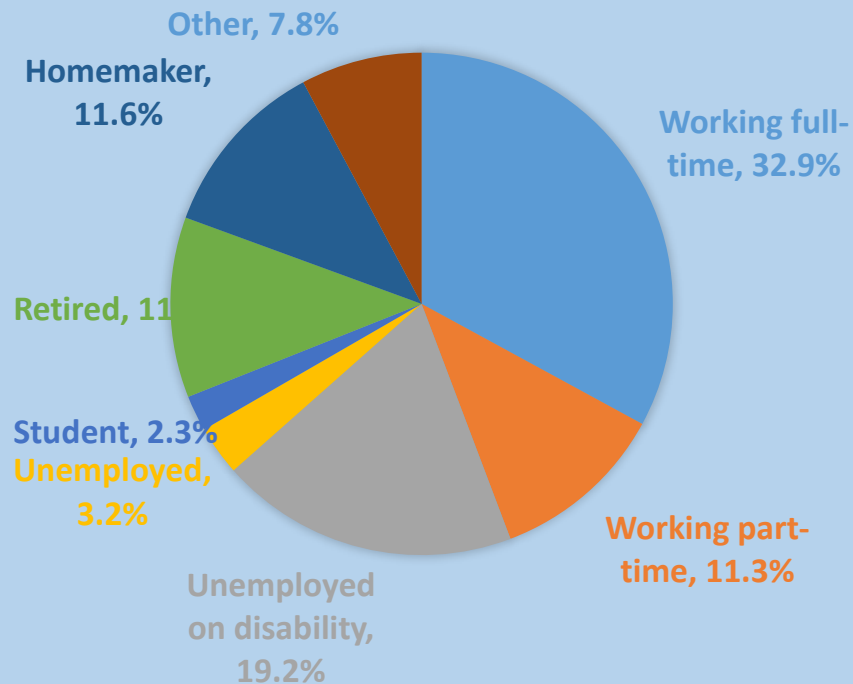


N=2995



Employment

What best describes your current employment?

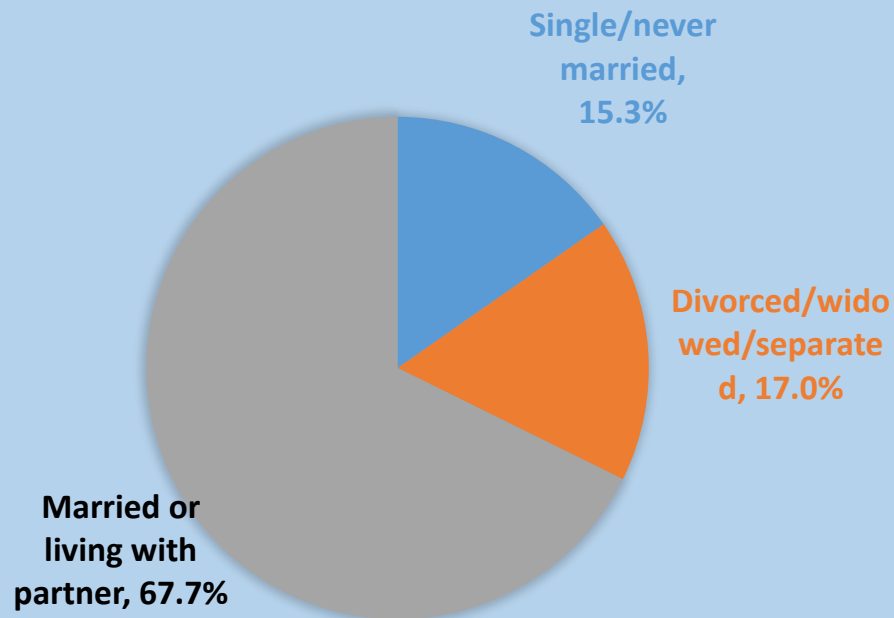


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Marital Status

Which of the following best describes your household situation?

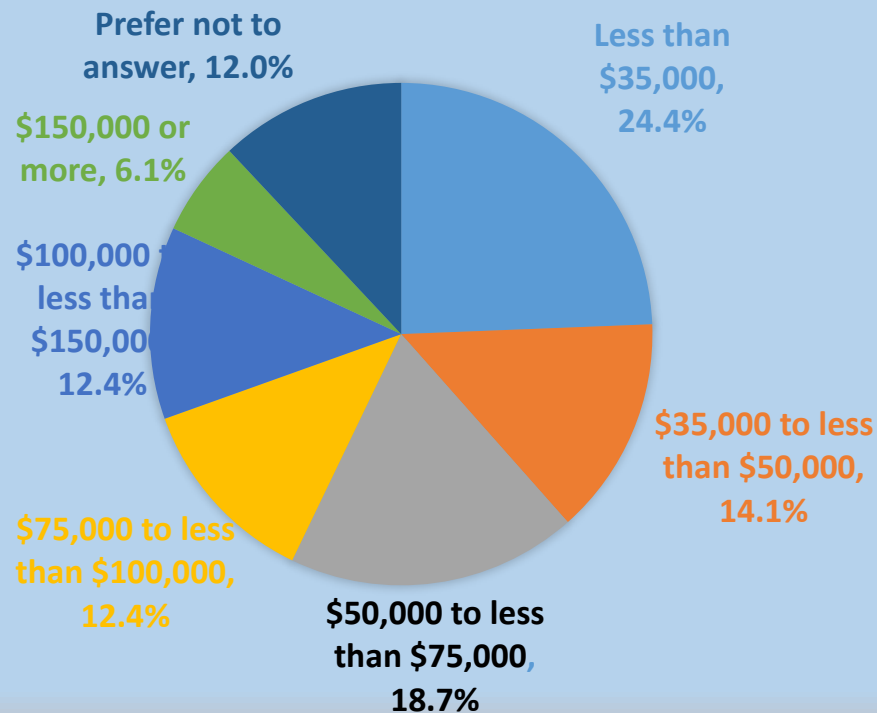


N=2991



Household Income

What is your total annual household income?



N=2987

