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Socio-economic burden of rare diseases: A systematic review of cost of illness evidence

DOI: 10.1016/j.healthpol.2014.12.016
Health Policy, 2015, 119, 964-79.

Source: <https://exaly.com/paper-pdf/62357514/citation-report.pdf>

Version: 2024-04-29

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167	Rare Diseases of the Anterior Segment of the Eye: Update on Diagnosis and Management. 2015 , 2015, 947326		2
166	A collaborative environment for shared classification of neuroimages: The experience of the Colibri project. 2015 , 2015, 4306-9		
165	Social and economic costs and health-related quality of life in non-institutionalised patients with cystic fibrosis in the United Kingdom. <i>BMC Health Services Research</i> , 2015 , 15, 428	2.9	31
164	The Genetic Basis of Mendelian Phenotypes: Discoveries, Challenges, and Opportunities. 2015 , 97, 199-215		432
163	Therapeutic potential of orphan drugs for the rare skeletal muscle diseases. 2015 , 3, 1397-1425		4
162	Socioeconomic costs and health-related quality of life in juvenile idiopathic arthritis: a cost-of-illness study in the United Kingdom. 2016 , 17, 321		16
161	Health Economic Data in Reimbursement of New Medical Technologies: Importance of the Socio-Economic Burden as a Decision-Making Criterion. <i>Frontiers in Pharmacology</i> , 2016 , 7, 252	5.6	6
160	The psychological burden of cystic fibrosis. 2016 , 22, 187-91		54
159	Economic burden and health-related quality of life associated with Prader-Willi syndrome in France. 2016 , 60, 879-90		10
158	Social/economic costs and health-related quality of life in patients with epidermolysis bullosa in Europe. 2016 , 17 Suppl 1, 31-42		25
157	Social/economic costs and health-related quality of life in patients with scleroderma in Europe. 2016 , 17 Suppl 1, 109-17		16
156	Social/economic costs and health-related quality of life in patients with Prader-Willi syndrome in Europe. 2016 , 17 Suppl 1, 99-108		17
155	Social/economic costs and health-related quality of life in patients with juvenile idiopathic arthritis in Europe. 2016 , 17 Suppl 1, 79-87		25
154	The High Direct Medical Costs of Prader-Willi Syndrome. 2016 , 175, 137-43		19

153	Opportunities and technical challenges in next-generation sequencing for diagnosis of rare pediatric diseases. 2016 , 16, 1073-1082		11
152	Discovery of new diketopiperazines inhibiting Burkholderia cenocepacia quorum sensing in vitro and in vivo. 2016 , 6, 32487		28
151	Social/economic costs and health-related quality of life of mucopolysaccharidosis patients and their caregivers in Europe. 2016 , 17 Suppl 1, 89-98		20
150	The Economic Burden of Childhood Glaucoma. 2016 , 25, 790-797		9
149	Epidermolysis Bullosa. 2016 , 8, 46-56		2
148	Cystic fibrosis on the African continent. <i>Genetics in Medicine</i> , 2016 , 18, 653-62	8.1	21
147	Development of prenatal screening--A historical overview. 2016 , 40, 12-22		44
146	Perceived economic burden associated with an inherited cardiac condition: a qualitative inquiry with families affected by arrhythmogenic right ventricular cardiomyopathy. <i>Genetics in Medicine</i> , 2016 , 18, 584-92	8.1	4
145	The burden, epidemiology, costs and treatment for Duchenne muscular dystrophy: an evidence review. <i>Orphanet Journal of Rare Diseases</i> , 2017 , 12, 79	4.2	202
144	Ethical imperatives of timely access to orphan drugs: is possible to reconcile economic incentives and patients' health needs?. <i>Orphanet Journal of Rare Diseases</i> , 2017 , 12, 1	4.2	67
143	Gene therapy for monogenic liver diseases: clinical successes, current challenges and future prospects. 2017 , 40, 497-517		54
142	Cost-of-Illness in Rare Diseases. 2017 , 1031, 283-297		3
141	Estimating Direct Cost of Cystic Fibrosis Care Using Irish Registry Healthcare Resource Utilisation Data, 2008-2012. 2017 , 35, 1087-1101		7
140	Review of 11 national policies for rare diseases in the context of key patient needs. <i>Orphanet Journal of Rare Diseases</i> , 2017 , 12, 63	4.2	75
139	A window into living with an undiagnosed disease: illness narratives from the Undiagnosed Diseases Network. <i>Orphanet Journal of Rare Diseases</i> , 2017 , 12, 71	4.2	28
138	Individual and family issues surrounding a rare disease. 2017 , 6, 45		1
137	The health and life path of rare disease patients: results of the 2015 French barometer. <i>Patient Related Outcome Measures</i> , 2017 , 8, 97-110	2.9	5
136	In silico clinical trials for pediatric orphan diseases. 2018 , 8, 2465		13

135	The price of abandoning diagnostic testing for cell-free fetal DNA screening. 2018 , 38, 243-245		20
134	Research in Rare Disease: From Genomics to Proteomics. 2018 , 16, 12-14		2
133	National Rare Diseases Registry System of China and Related Cohort Studies: Vision and Roadmap. 2018 , 29, 128-135		13
132	Psychosocial Profiles of Parents of Children with Undiagnosed Diseases: Managing Well or Just Managing?. 2018 , 27, 935-946		30
131	Impact of a rare chronic genodermatosis on family daily life: the example of epidermolysis bullosa. 2018 , 179, 1177-1178		1
130	Epidemiology of Sanfilippo syndrome: results of a systematic literature review. <i>Orphanet Journal of Rare Diseases</i> , 2018 , 13, 53	4.2	31
129	Patient reported outcome measures in rare diseases: a narrative review. <i>Orphanet Journal of Rare Diseases</i> , 2018 , 13, 61	4.2	73
128	Patients With Fragile X Syndrome Attending a Specialized Centre in Chile: Parent Satisfaction, Costs and Adherence. 2018 , 15, 63-69		1
127	The potential and benefits of repurposing existing drugs to treat rare muscular dystrophies. 2018 , 6, 259-271		4
126	A Systematic Review of Cost-of-Illness Studies of Multimorbidity. 2018 , 16, 15-29		66
125	Using multi-criteria decision analysis to appraise orphan drugs: a systematic review. 2018 , 18, 135-146		15
124	Mapping health care of rare diseases: the example of epidermolysis bullosa in Germany. <i>Orphanet Journal of Rare Diseases</i> , 2018 , 13, 197	4.2	4
123	Multi-Criteria Decision Analysis (MCDA) Models in Health Technology Assessment of Orphan Drugs-a Systematic Literature Review. Next Steps in Methodology Development?. 2018 , 6, 287		18
122	Planning a One Health Case Study to Evaluate Methicillin Resistant and Its Economic Burden in Portugal. 2018 , 9, 2964		8
121	The therapeutic potential of RNA regulation in neurological disorders. 2018 , 22, 1017-1028		12
120	The epidemic of abnormal copy number variant cases missed because of reliance upon noninvasive prenatal screening. 2018 , 38, 730-734		15
119	Direct and Indirect Costs of Immunoglobulin Replacement Therapy in Patients with Common Variable Immunodeficiency (CVID) and X-Linked Agammaglobulinemia (XLA) in Italy. 2018 , 38, 955-965		1
118	Characteristics of undiagnosed diseases network applicants: implications for referring providers. <i>BMC Health Services Research</i> , 2018 , 18, 652	2.9	11

117	Use of zebrafish models to investigate rare human disease. 2018 , 55, 641-649		21
116	Tailored frequency-escalated primary prophylaxis for severe haemophilia A: results of the 16-year Canadian Hemophilia Prophylaxis Study longitudinal cohort. 2018 , 5, e252-e260		26
115	Inhaled Biologicals for the Treatment of Cystic Fibrosis. 2019 , 13, 19-26		8
114	Orphan drugs <i>In der Epileptologie</i> . 2019 , 32, 277-285		3
113	Value of the Rare Disease Registry of the Italian Region Friuli Venezia Giulia. 2019 , 22, 1003-1011		2
112	Periodic Fever, Aphthous Stomatitis, Pharyngitis, and Cervical Adenitis Syndrome (PFAPA): A Clinical Challenge for Primary Care Physicians and Rheumatologists. 2019 , 7, 277		7
111	Point of Care Exome Sequencing Reveals Allelic and Phenotypic Heterogeneity Underlying Mendelian disease in Qatar. 2019 , 28, 3970-3981		5
110	Analysis of economic burden and its associated factors of twenty-three rare diseases in Shanghai. <i>Orphanet Journal of Rare Diseases</i> , 2019 , 14, 233	4.2	6
109	A cost of illness study evaluating the burden of Wolfram syndrome in the United Kingdom. <i>Orphanet Journal of Rare Diseases</i> , 2019 , 14, 185	4.2	3
108	Clinical research challenges in rare genetic diseases in Brazil. 2019 , 42, 305-311		5
107	[Complex care, high cost, and loss of income: frequent issues for families of children and adolescents with rare health conditions]. 2019 , 35, e00180218		3
106	How to Advance Research, Education, and Training in the Study of Rare Diseases. 2019 , 157, 917-921		5
105	Tumor-associated macrophages: role in cancer development and therapeutic implications. 2019 , 42, 591-608		79
104	Seeking the state of the art in standardized measurement of health care resource use and costs in juvenile idiopathic arthritis: a scoping review. <i>Pediatric Rheumatology</i> , 2019 , 17, 20	3.5	5
103	Cost-of-illness studies in nine Central and Eastern European countries. 2019 , 20, 155-172		13
102	The Economic Burden of Cystic Fibrosis in Germany from a Payer Perspective. 2019 , 37, 1029-1039		7
101	The combination of tezacaftor and ivacaftor in the treatment of patients with cystic fibrosis: clinical evidence and future prospects in cystic fibrosis therapy. 2019 , 13, 1753466619844424		15
100	The burden of rare diseases. 2019 , 179, 885-892		70

99	The patient's view on rare disease trial design - a qualitative study. <i>Orphanet Journal of Rare Diseases</i> , 2019 , 14, 31	4.2	16
98	The cumulative incidence and trends of rare diseases in South Korea: a nationwide study of the administrative data from the National Health Insurance Service database from 2011-2015. <i>Orphanet Journal of Rare Diseases</i> , 2019 , 14, 49	4.2	19
97	Rare diseases in Chile: challenges and recommendations in universal health coverage context. <i>Orphanet Journal of Rare Diseases</i> , 2019 , 14, 289	4.2	9
96	Adult Care in Cystic Fibrosis. 2019 , 40, 857-868		3
95	Experiences of patients with Poland syndrome of diagnosis and care in Italy: a pilot survey. <i>Orphanet Journal of Rare Diseases</i> , 2019 , 14, 269	4.2	10
94	Comment on "The price of abandoning diagnostics testing for cell-free fetal DNA screening". 2019 , 39, 130		
93	Direct health-care costs for children diagnosed with genetic diseases are significantly higher than for children with other chronic diseases. <i>Genetics in Medicine</i> , 2019 , 21, 1049-1057	8.1	7
92	HTA programme response to the challenges of dealing with orphan medicinal products: Process evaluation in selected European countries. <i>Health Policy</i> , 2019 , 123, 140-151	3.2	26
91	Economics of dementia: A review of methods. 2020 , 19, 1426-1440		5
90	A Nationwide Study of Prevalence Rates and Characteristics of 199 Chronic Conditions in Denmark. 2020 , 4, 361-380		23
89	The changing landscape of clinical trials for mitochondrial diseases: 2011 to present. 2020 , 50, 51-57		4
88	Societal costs and burden of hereditary transthyretin amyloidosis polyneuropathy. 2020 , 27, 89-96		2
87	Rare Diseases: Genomics and Public Health. 2020 , 37-51		0
86	Pharmacogenomic phase transition from personalized medicine to patient-centric customized delivery. 2020 , 20, 1-18		8
85	The Impact of Next-Generation Sequencing on the Diagnosis, Treatment, and Prevention of Hereditary Neuromuscular Disorders. 2020 , 24, 641-652		0
84	Hospital incidence, management and direct cost of osteogenesis imperfecta in Spain: a retrospective database analysis. 2020 , 23, 1435-1440		1
83	The urgent need to empower rare disease organizations in China: an interview-based study. <i>Orphanet Journal of Rare Diseases</i> , 2020 , 15, 282	4.2	6
82	Recalibrating Health Technology Assessment Methods for Cell and Gene Therapies. 2020 , 38, 1297-1308		13

81	Prenatal Testing or Screening?. 2020 , 2, 217-222		1
80	Next Generation Sequencing and Bioinformatics Analysis of Family Genetic Inheritance. 2020 , 11, 544-162		11
79	[Interdisciplinary care of patients with short-bowel-syndrome in Germany: A comparative Cost-of-Illness analysis of in- and outpatient care]. 2020 , 58, 364-372		
78	CFTR Modulators: The Changing Face of Cystic Fibrosis in the Era of Precision Medicine. <i>Frontiers in Pharmacology</i> , 2019 , 10, 1662	5.6	112
77	Challenges Affecting Access to Health and Social Care Resources and Time Management among Parents of Children with Rett Syndrome: A Qualitative Case Study. <i>International Journal of Environmental Research and Public Health</i> , 2020 , 17,	4.6	3
76	Cost of early-stage mycosis fungoides treatments in Spain. 2020 , 12, 91-105		
75	The role of primary care in management of rare diseases in Ireland. 2020 , 189, 771-776		3
74	The cost of hemophilia treatment: the importance of minimizing it without detriment to its quality. 2020 , 13, 269-274		7
73	Mining Complex Biomedical Literature for Actionable Knowledge on Rare Diseases. 2020 , 77-94		1
72	Outcome measures in Haemophilia: Beyond ABR (Annualized Bleeding Rate). 2021 , 27 Suppl 3, 87-95		8
71	Reflections on the Importance of Cost of Illness Analysis in Rare Diseases: A Proposal. <i>International Journal of Environmental Research and Public Health</i> , 2021 , 18,	4.6	1
70	The Odyssey of Rare Disease Patients: Navigating the Pathways to Diagnosis. <i>SSRN Electronic Journal</i> ,	1	
69	Impact of single and combined rare diseases on adult inpatient outcomes: a retrospective, cross-sectional study of a large inpatient population. <i>Orphanet Journal of Rare Diseases</i> , 2021 , 16, 105	4.2	0
68	Systematic assessment of outcomes following a genetic diagnosis identified through a large-scale research study into developmental disorders. <i>Genetics in Medicine</i> , 2021 , 23, 1058-1064	8.1	1
67	Towards a definition of Ancient Rare Diseases (ARD): Presenting a complex case of probable Legg-Calvé-Perthes Disease from the North Caucasian Bronze Age (2200-1650 cal BCE). <i>International Journal of Paleopathology</i> , 2021 , 32, 61-73	1.5	1
66	Cost-of-illness studies in rare diseases: a scoping review. <i>Orphanet Journal of Rare Diseases</i> , 2021 , 16, 178	4.2	3
65	A multi-stakeholder multicriteria decision analysis for the reimbursement of orphan drugs (FinMHU-MCDA study). <i>Orphanet Journal of Rare Diseases</i> , 2021 , 16, 186	4.2	0
64	New ethical challenges in the management of rare pediatric diseases with innovative therapies. <i>Archives De Pediatrie</i> , 2021 , 28, 311-318	1.8	0

63	Methods for Estimating Avoidable Costs of Excessive Alcohol Consumption. <i>International Journal of Environmental Research and Public Health</i> , 2021 , 18,	4.6	3
62	Functional Genomics for Undiagnosed Patients: The Impact of Small GTPases Signaling Dysregulation at Pan-Embryo Developmental Scale. <i>Frontiers in Cell and Developmental Biology</i> , 2021 , 9, 642235	5.7	0
61	Healthcare Resource Consumption and Cost of Invasive Meningococcal Disease in France: A Study of the National Health Insurance Database. <i>Infectious Diseases and Therapy</i> , 2021 , 10, 1607-1623	6.2	5
60	Can you hear us now? The impact of health-care utilization by rare disease patients in the United States. <i>Genetics in Medicine</i> , 2021 , 23, 2194-2201	8.1	6
59	Left to themselves: Time to target chronic pain in childhood rare diseases. <i>Neuroscience and Biobehavioral Reviews</i> , 2021 , 126, 276-288	9	0
58	Identifying Potential Gamification Elements for A New Chatbot for Families with Neurodevelopmental Disorders: A User-Centred Design Approach (Preprint).		
57	Experiences of parents with children with congenital disorders of glycosylation: What can we learn from them?. <i>Disability and Health Journal</i> , 2021 , 14, 101065	4.2	1
56	Navigating the U.S. health insurance landscape for children with rare diseases: a qualitative study of parents' experiences. <i>Orphanet Journal of Rare Diseases</i> , 2021 , 16, 313	4.2	3
55	Gene-Targeted Therapies in Pediatric Neurology: Challenges and Opportunities in Diagnosis and Delivery. <i>Pediatric Neurology</i> , 2021 , 125, 53-57	2.9	1
54	Biologic medicine inclusion in 138 national essential medicines lists. <i>Pediatric Rheumatology</i> , 2021 , 19, 140	3.5	
53	Health economic benefits through the use of diagnostic support systems and expert knowledge. <i>BMC Health Services Research</i> , 2021 , 21, 947	2.9	2
52	Aquatic therapy for children with Duchenne muscular dystrophy: a pilot feasibility randomised controlled trial and mixed-methods process evaluation. <i>Health Technology Assessment</i> , 2017 , 21, 1-120	4.4	10
51	Gene therapy for inherited metabolic diseases. <i>Medycyna Wieku Rozwojowego</i> , 2020 , 24, 53-64	0.4	3
50	The IDeaS initiative: pilot study to assess the impact of rare diseases on patients and healthcare systems. <i>Orphanet Journal of Rare Diseases</i> , 2021 , 16, 429	4.2	6
49	Labor market participation and productivity costs for female caregivers of minor male children with Duchenne and Becker muscular dystrophies. <i>Muscle and Nerve</i> , 2021 , 64, 717-725	3.4	0
48	Proposing a Core Outcome Set for Physical Activity and Exercise Interventions in People With Rare Neurological Conditions. <i>Frontiers in Rehabilitation Sciences</i> , 2021 , 2,		0
47	Commentary: Individuals affected by Eosinophilic Gastrointestinal Disorders Have Complex Unmet Needs and Experience Barriers to Care. <i>Journal of Rare Diseases Research & Treatment</i> , 2018 , 3, 34-36	1.1	0
46	Clinical genetics and its adjacent regimes.		1

45	PROBLEMATIC ISSUES OF ORPHAN DISEASES OF THE NERVOUS SYSTEM IN CHILDREN. <i>Journal of the National Academy of Medical Sciences of Ukraine</i> , 2021 , 140-148	0.2	
44	Systematic assessment of outcomes following a genetic diagnosis identified through a large-scale research study into developmental disorders.		
43	Economic Burden of Rare Diseases With Common Diseases as a Comorbidity in Poland. 2020 , 2/2020, 103-120	0.2	
42	Pulmonary Function and Hospital Admission in Patients with Cystic Fibrosis Based on Household Second-Hand Smoking. <i>Tanaffos</i> , 2018 , 17, 37-41	0.5	1
41	An Analysis of Medical Care Services for Children With Rare Diseases in the Russian Federation.. <i>Frontiers in Pharmacology</i> , 2021 , 12, 754073	5.6	2
40	Seltene Erkrankungen Ein Überblick. 2021 , 1-12		1
39	Economic Burden of Epidermolysis Bullosa Disease in Iran.. <i>Medical Journal of the Islamic Republic of Iran</i> , 2021 , 35, 146	1.1	
38	Healthcare resource utilisation and medical costs for children with interstitial lung diseases (chILD) in Europe.. <i>Thorax</i> , 2022 ,	7.3	0
37	The rare disease neurofibromatosis 1 as a source of hereditary economic inequality: Evidence from Finland.. <i>Genetics in Medicine</i> , 2021 ,	8.1	0
36	Do Dermatological Diseases Cause Disability? A Single Tertiary Center Experience. 2021 , 0-0		
35	Key challenges for hub and spoke models of care A report from the 1st workshop of the EHC Think Tank on Hub and Spoke Treatment Models. <i>The Journal of Haemophilia Practice</i> , 2022 , 9, 20-26	0.2	
34	Genomic approaches to improve the clinical diagnosis and management of patients with congenital hydrocephalus. <i>Journal of Neurosurgery: Pediatrics</i> , 2021 , 1-10	2.1	0
33	Mental Health of Siblings of Children with Rare Congenital Surgical Diseases during the COVID-19 Pandemic.. <i>European Journal of Pediatric Surgery</i> , 2021 ,	1.9	
32	Work Outcomes Among Patients with Light Chain (AL) Amyloidosis: Findings from Three Patient Cohorts.. <i>Patient Related Outcome Measures</i> , 2021 , 12, 339-347	2.9	
31	Quality of life in neurofibromatosis 1: development and validation of a tool dedicated to cutaneous neurofibromas in adults.. <i>Journal of the European Academy of Dermatology and Venereology</i> , 2022 ,	4.6	
30	The national economic burden of rare disease in the United States in 2019.. <i>Orphanet Journal of Rare Diseases</i> , 2022 , 17, 163	4.2	3
29	Designing rare disease care pathways in the Republic of Ireland: a co-operative model.. <i>Orphanet Journal of Rare Diseases</i> , 2022 , 17, 162	4.2	
28	Recommendations from the IRDiRC Working Group on methodologies to assess the impact of diagnoses and therapies on rare disease patients.. <i>Orphanet Journal of Rare Diseases</i> , 2022 , 17, 181	4.2	0

27	Remote cognitive training for children with congenital brain malformation or genetic syndrome: a scoping review.. <i>Journal of Intellectual Disabilities</i> , 2022 , 17446295221095712	1.8	
26	Evaluation of Quality of Life in Patients with Cystic Fibrosis Regarding Smoking History in Parents. <i>Journal of Comprehensive Pediatrics</i> , 2022 , 13,	0.7	
25	Diagnostic journeys: characterization of patients and diagnostic outcomes from an academic second opinion clinic. <i>Diagnosis</i> , 2022 ,	4.2	0
24	Langerhans Cell Histiocytosis. <i>Evidence-based Imaging</i> , 2022 , 1-17		
23	Diagnostic uncertainty in patients, parents, and physicians: a compensatory control theory perspective. <i>Health Psychology Review</i> , 1-17	7.1	0
22	Healthcare Burden of Rare Diseases: A Population-Based Study in Tuscany (Italy). <i>International Journal of Environmental Research and Public Health</i> , 2022 , 19, 7553	4.6	0
21	Valuing the Burden and Impact of Rare Diseases: A Scoping Review. <i>Frontiers in Pharmacology</i> , 13,	5.6	2
20	Identifying Potential Gamification Elements for A New Chatbot for Families with Neurodevelopmental Disorders: A User-Centred Design Approach (Preprint). <i>JMIR Human Factors</i> ,	2.5	
19	Prevalence, clinical features, and outcomes of COVID-19 in persons with cystic fibrosis: a systematic review protocol. Publish Ahead of Print,		
18	Use of Netnography to Understand GoFundMe Crowdfunding Profiles Posted for Individuals and Families of Children with Osteogenesis Imperfecta. 2022 , 10, 1451		1
17	GENE TARGET: A framework for evaluating Mendelian neurodevelopmental disorders for gene therapy. 2022 , 27, 32-46		1
16	Understanding the socioeconomic costs of dystrophic epidermolysis bullosa in Europe: a costing and health-related quality of life study. 2022 , 17,		0
15	Catalogue of multimorbidity mean based severity and associational prevalence rates between 199+ chronic conditionsA nationwide register-based population study. 2022 , 17, e0273850		1
14	The genetic determinants of oral diseases in Africa: The gaps should be filled. 3,		0
13	The Tumor Microenvironment. 2022 , 1-49		0
12	Lungenerkrankungen. 2022 , 289-378		0
11	Profile of Drug Utilization in Patients with Rare Diseases in Tuscany, Italy: A Population-Based Study. 2023 , 20, 937		0
10	Catalogue of socioeconomic disparities and characteristics of 199+ chronic conditionsA nationwide register-based population study. 2022 , 17, e0278380		1

- 9 Socio-economic costs of rare diseases and the risk of financial hardship: a cross-sectional study. **2023**, 100711
- 8 Enhancing Equitable Access to Rare Disease Diagnosis and Treatment around the World: A Review of Evidence, Policies, and Challenges. **2023**, 20, 4732
- 7 Congenital Athymia: Unmet Needs and Practical Guidance. Volume 19, 239-254
- 6 Effect of the copayment reduction system on accessibility to orphan drugs in South Korea. 1-7
- 5 Review of economic modeling evidence from NICE appraisals of rare disease treatments for spinal muscular atrophy. 1-14
- 4 Two Cases of Myofibrillar Myopathies: Genetic and Quality of Life Study. **2023**, 2, 177-186
- 3 Developing a Framework of Cost Elements of Socioeconomic Burden of Rare Disease: A Scoping Review.
- 2 What is the awareness of rare diseases among medical students? A survey in Bulgaria.
- 1 Challenges of Clinical Research in Orphan Diseases. **2023**, 11-21