

# CITATION REPORT

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## Recommendations for the design of small population clinical trials

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#	Paper	IF	Citations
52	Executive summary of the NHLBI State of the Science (SOS) Workshop: Overview and next steps in generating a national blueprint for future research on factor VIII inhibitors. <i>Haemophilia</i> , <b>2019</b> , 25, 610-613	3.3	5
51	Inherited Retinal Disorders: Using Evidence as a Driver for Implementation. <i>Ophthalmologica</i> , <b>2019</b> , 242, 187-194	3.7	10
50	Efficacy and Safety of Dichlorphenamide for Primary Periodic Paralysis in Adolescents Compared With Adults. <i>Pediatric Neurology</i> , <b>2019</b> , 101, 43-46	2.9	1
49	Clinical-Pharmacogenetic Predictive Models for Time to Occurrence of Levodopa Related Motor Complications in Parkinson's Disease. <i>Frontiers in Genetics</i> , <b>2019</b> , 10, 461	4.5	10
48	Dual Role of but Not Inflammasome Polymorphisms in Type 1 Diabetes and Celiac Disease in Children. <i>Frontiers in Pediatrics</i> , <b>2019</b> , 7, 63	3.4	4
47	Artificial Intelligence (AI) in Rare Diseases: Is the Future Brighter?. <i>Genes</i> , <b>2019</b> , 10,	4.2	32
46	Orphan Medicines for Pediatric Use: A Focus on the European Union. <i>Clinical Therapeutics</i> , <b>2019</b> , 41, 2630-2642	3.3	3
45	Orphan drugs: major development challenges at the clinical stage. <i>Drug Discovery Today</i> , <b>2019</b> , 24, 867-878	3.8	14
44	Access to Affordable Orphan Medicines in Europe: An EHA Position Paper. <i>HemaSphere</i> , <b>2020</b> , 4, e477	0.3	2
43	Translational perspectives to treat Epidermolysis bullosa-Where do we stand?. <i>Experimental Dermatology</i> , <b>2020</b> , 29, 1112-1122	4	6
42	Profiling trial burden and patients' attitudes to improve clinical research in epidermolysis bullosa. <i>Orphanet Journal of Rare Diseases</i> , <b>2020</b> , 15, 182	4.2	6
41	Excisional treatment comparison for in situ endocervical adenocarcinoma (EXCISE): A phase 2 pilot randomized controlled trial to compare histopathological margin status, specimen size and fragmentation after loop electrosurgical excision procedure and cold knife cone biopsy. <i>Gynecologic Oncology</i> , <b>2020</b> , 159, 623-629	4.9	1
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39	Clinical development of cell therapies for cancer: The regulators' perspective. <i>European Journal of Cancer</i> , <b>2020</b> , 138, 41-53	7.5	3
38	Precision Child Health: an Emerging Paradigm for Paediatric Quality and Safety. <i>Current Treatment Options in Pediatrics</i> , <b>2020</b> , 6, 317-324	0.6	1
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36	Market access of gene therapies across Europe, USA, and Canada: challenges, trends, and solutions. <i>Drug Discovery Today</i> , <b>2021</b> , 26, 399-415	8.8	7

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34	N-of-1 Trials in Rare Genetic Neurodevelopmental Disorders: Opportunities for Improvement. <i>Neurology</i> , <b>2021</b> , 96, 513-514	6.5	3
33	Rare Diseases and Otorhinolaryngology, Head and Neck Surgery. <i>Laryngo- Rhino- Otologie</i> , <b>2021</b> , 100, S1-S11	0.8	
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30	Validating online approaches for rare disease research using latent class mixture modeling. <i>Orphanet Journal of Rare Diseases</i> , <b>2021</b> , 16, 209	4.2	1
29	The efficacy and safety of setmelanotide in individuals with Bardet-Biedl syndrome or Alström syndrome: Phase 3 trial design. <i>Contemporary Clinical Trials Communications</i> , <b>2021</b> , 22, 100780	1.8	5
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18	Methodological Characteristics of Clinical Trials Supporting the Marketing Authorisation of Advanced Therapies in the European Union.. <i>Frontiers in Pharmacology</i> , <b>2021</b> , 12, 773712	5.6	2

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16	Epilepsy in patients with schizophrenia: Pathophysiology and basic treatments.. <i>Epilepsy and Behavior</i> , <b>2022</b> , 127, 108520	3.2	0
15	Design and analysis features used in small population and rare disease trials: A targeted review.. <i>Journal of Clinical Epidemiology</i> , <b>2021</b> ,	5.7	1
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11	Application of Bayesian methods to accelerate rare disease drug development: scopes and hurdles.. <i>Orphanet Journal of Rare Diseases</i> , <b>2022</b> , 17, 186	4.2	1
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5	Generalized pairwise comparisons of prioritized outcomes are a powerful and patient-centric analysis of multi-domain scores.		0
4	An evaluation of setmelanotide injection for chronic weight management in adult and pediatric patients with obesity due to BardetBiedl syndrome. 1-8		0
3	Study design challenges and strategies in clinical trials for rare diseases: Lessons learned from pantothenate kinase-associated neurodegeneration. 14,		0
2	Computational drug repositioning of clopidogrel as a novel therapeutic option for focal segmental glomerulosclerosis. <b>2023</b> ,		0
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