MarketVue® Sorbitol Dehydrogenase (SORD) Deficiency

March 2022



MarketVue®: SORD Deficiency

UNDERSTAND THE SORD DEFICIENCY MARKET

MarketVue market landscape reports combine primary (KOL interviews) and secondary market research to empower strategic decision-making and provide a complete view of the market.

Every MarketVue includes a disease overview, epidemiology (US), current treatment, unmet needs, pipeline and access and reimbursement chapter.

Methodology: Research is supported by 8 qualitative interviews with key opinion leaders (U.S. Neurologists specializing in Neuromuscular disorders) and secondary research.

Geographies covered: United States

ш ď ш 000 S EPIDEMIOLOGY: Understand prevalence, diagnosed and drug-treated prevalence of the population and key market segments

CURRENT TREATMENT: Understand the treatment decision tree and strengths and weaknesses of current on-label and off-label treatment

UNMET NEEDS: Identify opportunities to address treatment or disease management gaps

PIPELINE ANALYSIS: Compare current and emerging therapy clinical development strategy; their performance on efficacy, safety, and delivery metrics; and their potential to address unmet needs

VALUE AND ACCESS: Review the evidence needed to assess and communicate value to key stakeholders (e.g., providers, payers, regulators) and learn what competitors have done or are doing

Why MarketVue?

- PMR-Driven Insights informed by qualitative interviews and/or quantitative surveys
- Senior Team Experienced team members (10+ years in pharma market research) lead the research
- **Strategic –** Delivered in a concise and strategic report template vetted by pharmaceutical industry professionals
- Fresh New reports or report refreshes delivered in as little as 15 business days





MarketVue®: SORD Deficiency

UNDERSTAND THE SORD DEFICIENCY MARKET

COMPANIES MENTIONED

Applied Therapeutics

DRUGS MENTIONED

AT-007



MarketVue®: SORD Deficiency Table of Contents

1. DISEASE OVERVIEW	5 - 6
SORD Deficiency awareness	5
Figure 1.1. Neuromuscular specialist familiarity with SORD Deficiency	5
Figure 1.2. Percentage of neurologists familiar with role of sorbitol in periphe neuropathy	ral 5
Grading disease severity in CMT	6
Figure 1.3. Proportion of physicians using CMT severity scales to grade disease severity	6
Table 1.1. Description and utility of validated CMT disease severity scoring scale	6
2. EPIDEMIOLOGY & PATIENT POPULATIONS	7 - 8
Prevalence of SORD Deficiency	7
Figure 2.1. SORD Deficiency prevalence and population size in the United States, 2022	7
Figure 2.2. Global prevalence estimates of CMT, 1974 - 2019	7
Patient flow calculation of the number of SORD Deficiency patients	7
Figure 2.3. Derivation of SORD Deficiency population size in the United State 2022	es, 8
Figure 2.4. SORD Deficiency population segmented by disease severity	8
3. DIAGNOSIS & CURRENT TREATMENT	9 - 14
Diagnosis overview	9
Figure 3.1. Diagnosis flow CMT2 and SORD Deficiency patients	9
Delayed diagnosis of CMT disorders	10
Figure 3.2. Drivers of delayed diagnosis in CMT2 patients	10
Genetic testing in CMT	10
Figure 3.3. Genetic testing use and results in CMT2 patients	11
Figure 3.4. Summary of Invitae's Comprehensive Neuropathies Panel	11
Genetic re-testing for SORD Deficiency	12
Figure 3.5. Percentage of neuromuscular specialists who re-test patients for newly identified genes	12
Figure 3.6. Neuromuscular specialists' use of sorbitol testing now and in the	12



MarketVue®: SORD Deficiency Table of Contents

I reatment overview	13
Figure 3.7. Treatment goals for CMT2 disorders	13
Table 3.1. Standard of care – upside and downside	13
Treatment dynamics in CMT2	14
Table 3.2. Key CMT2 treatment dynamics that will impact SORD Deficiency now and in the future	14
4. UNMET NEED	15
Overview	15
Figure 4.1. Physician-reported unmet needs in CMT2 patients	15
5. PIPELINE ANALYSIS	16
Overview	16
Table 5.1 Summary of AT-007 INSPIRE study	16
Figure 5.1 Key considerations for emerging therapies for SORD Deficiency	16
6. VALUE & ACCESS	17-20
Insurance coverage of CMT2 patients	17
Insurance coverage of CMT2 patients Overview of Drug Analogues	17 17
Overview of Drug Analogues Figure 6.1. Average percentage of CMT patients covered by insurance type	17
Overview of Drug Analogues Figure 6.1. Average percentage of CMT patients covered by insurance type among interviewed physicians	17 17
Overview of Drug Analogues Figure 6.1. Average percentage of CMT patients covered by insurance type among interviewed physicians Table 6.1. Criteria for selecting SORD Deficiency analogues	17 17 17
Overview of Drug Analogues Figure 6.1. Average percentage of CMT patients covered by insurance type among interviewed physicians Table 6.1. Criteria for selecting SORD Deficiency analogues Commercial payer requirement of drug analogues Table 6.2. Summary of commercial insurance requirements for Soliris and	17 17 17 18
Overview of Drug Analogues Figure 6.1. Average percentage of CMT patients covered by insurance type among interviewed physicians Table 6.1. Criteria for selecting SORD Deficiency analogues Commercial payer requirement of drug analogues Table 6.2. Summary of commercial insurance requirements for Soliris and Oxlumo Figure 6.2. Important market access considerations for SORD Deficiency drug	17 17 17 18 18
Overview of Drug Analogues Figure 6.1. Average percentage of CMT patients covered by insurance type among interviewed physicians Table 6.1. Criteria for selecting SORD Deficiency analogues Commercial payer requirement of drug analogues Table 6.2. Summary of commercial insurance requirements for Soliris and Oxlumo Figure 6.2. Important market access considerations for SORD Deficiency drug developers	17 17 17 18 18
Overview of Drug Analogues Figure 6.1. Average percentage of CMT patients covered by insurance type among interviewed physicians Table 6.1. Criteria for selecting SORD Deficiency analogues Commercial payer requirement of drug analogues Table 6.2. Summary of commercial insurance requirements for Soliris and Oxlumo Figure 6.2. Important market access considerations for SORD Deficiency drug developers Alnylam's market access pillars for Oxlumo Figure 6.3. Alnylam market access strategies to promote access to Oxlumo in	17 17 17 18 18 18
Overview of Drug Analogues Figure 6.1. Average percentage of CMT patients covered by insurance type among interviewed physicians Table 6.1. Criteria for selecting SORD Deficiency analogues Commercial payer requirement of drug analogues Table 6.2. Summary of commercial insurance requirements for Soliris and Oxlumo Figure 6.2. Important market access considerations for SORD Deficiency drug developers Alnylam's market access pillars for Oxlumo Figure 6.3. Alnylam market access strategies to promote access to Oxlumo in the United States	17 17 17 18 18 18 19



MarketVue®: SORD Deficiency Table of Contents

7. METHODOLOGY	21-24
Primary Market Research Approach	21
Table 7.1. Participant Screening Criteria	21
Figure 7.1. Interviewed neuromuscular specialists segmented by types of patients seen (number of physicians)	21
Diagnosed Prevalence Estimates Approach	22
Disease Definition	22
Table 7.3. Key Population Dynamics References	22
Table 7.4. CMT Prevalence References	23
Table 7.5. dHMN Prevalence References	24
Table 7.6. CMT Subtype Prevalence References	24



Meet the REACH Team







MELISSA CURRAN is the Director of Product Management at REACH. Melissa has over 10 years of life sciences market research and consulting experience spanning from bespoke strategy consulting to syndicated market research product development and management. Prior to joining REACH, she worked at Decision Resources Group (DRG) for 7 years assisting pharmaceutical and biotechnology commercial teams across the product lifecycle to inform strategic decision making. Melissa is particularly passionate about new product planning and portfolio management, especially in the rare disease space where data can be scarce, and decision-making can be challenging. Specific types of strategic assessments Melissa specializes in include market landscape assessments, commercial opportunity assessment, patient journey mapping, product positioning and TPP optimization, portfolio prioritization, and competitive intelligence. She also has extensive experience working across various market research methodologies including qualitative interviews, quantitative surveys, patient chart audits, real world claims and EHR data, conjoint analysis and secondary research. Melissa received her bachelor's degree in Biology and minor in Business from Providence College.



MICHAEL HUGHES, MSc, Ph.D., Dr. Hughes is the Director of Research at REACH. He has worked in academia, regulatory affairs (NICE) and in RWE and epidemiology consultancies, leading the global epidemiology team at Clarivate (previously Decision Resources Group) for many years. Over that period, he has built numerous new approaches to epidemiological forecasting and imputation, which now form industry best-practice. He has built syndicated and custom epidemiological models and forecasts for many blockbuster drugs across many therapeutic areas, often using a hybrid approach sourcing data from multiple types of dataset and primary market research. He has recently worked on projects in prostate cancer, amyloidosis, anaphylaxis and multi-drug resistant UTIs, among others. He has supported the needs of both big pharma, including Novartis, GSK and Johnson and Johnson, as well as smaller companies and biotechs.



Meet the REACH Team



TYLER JAKAB, MPH is an analyst at REACH Market Research. He is responsible for conducting both primary and secondary market research regarding rare disease therapies to be integrated into market research reports for life science clients. Tyler is a recent graduate of Boston University School of Public Health where he obtained an MPH in Epidemiology of Biostatistics. Prior to joining REACH, he held roles in which he was responsible for health policy analysis, tobacco control research, and health communication. He has extensive experience in data analysis, as well as manuscript and report writing. Tyler also earned a BS in Psychology and Anthropology from the University of North Carolina at Chapel Hill.



BAYLEY KOOPMAN is a Research Associate at REACH Market Research. At REACH, Bayley supports both primary and secondary market research through literature reviews and working with qualitative data. He recently graduated from Tufts University with a B.S. in Biology where he studied the interdisciplinary OneHealth approach for public health and the environment. During this time, Bayley founded an early-stage consumer product startup, which became a finalist team in two consecutive Tufts University Entrepreneurship Pitch Competitions. Prior to joining REACH, Bayley also held roles in regulatory affairs in the rare-disease pharmaceutical industry and veterinary practice.



BRIANA MULLINS is a current PhD student At NYU School of Medicine studying the immunological progression of disease in psoriatic arthritis. She currently does both laboratory research and computational biology. Previously she earned her undergraduate degree in Biochemistry at New York University (NYU) and worked in the Blaser Lab studying the human microbiome. She also received an MSc. in Population Health at the University College London (UCL) and conducted antibiotic prescription research using the UK THIN Database. Before starting her PhD Briana worked at Decision Resources Group as an Associate Epidemiologist.

