

MarketVue®

Angelman Syndrome

February 2024



MarketVue®: Angelman Syndrome

UNDERSTAND THE ANGELMAN SYNDROME 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 and EU5), current treatment, unmet needs, pipeline and access and reimbursement chapter.

Methodology: Research is supported by 8 qualitative interviews with key opinion leaders and secondary research. Participants included 1 Psychiatrist, 1 Psychologist, 1 Pediatric Geneticist, 3 Pediatric Neurologists, 1 Pediatric Epileptologist, and 1 Developmental Pediatrician (all U.S.).

Geographies covered: United States plus epidemiology for EU5 (France, Germany, Italy, Spain, United Kingdom)

TOPICS COVERED

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



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COMPANIES MENTIONED

- Hoffman-La Roche
- Ionis Pharmaceuticals
- Biogen
- Ultragenyx Pharmaceutical
- Neuren Pharmaceuticals
- Foundation for Angelman Syndrome Therapeutics (FAST)
- Encoded Therapeutics
- Transformatx Biotherapeutics
- Ovid Therapeutics

DRUGS MENTIONED

- Melatonin
- Trazodone
- Clonazepam
- Lamotrigine
- Levetiracetam
- Topiramate
- Clonidine
- Atomoxetine (Strattera)
- Risperidone
- Aripiprazole (Abilify)
- Polyethylene glycol
- Omeprazole
- Rugonersen
- ION582
- GTX-102
- NNZ-2591
- Alogabat
- GTP-220
- Gaboxadol

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Meet the REACH Team



DANIELLE DRAYTON, PhD., Dr. Drayton is CEO and Founder of REACH Market Research. She is a seasoned business leader and pharmaceutical market researcher. Prior to founding REACH, Dr. Drayton led the Biopharma Market Assessment business at Decision Resources Group (DRG) comprised of market research, RWD analytics, and consulting business lines. In her 14 years at DRG, she worked with 48 of the top 50 pharmaceutical companies and countless biotech companies that involved exhaustive evaluation of unmet need, target product profiles, commercial potential and new product adoption, and company competitiveness. She also has extensive experience conducting product and market opportunity assessments, portfolio analysis, product and therapeutic area strategy, product valuation and sales forecasting, and pre-launch planning. Dr. Drayton completed a postdoctoral fellowship at the Harvard Medical School, received a Ph.D. in Immunobiology from Yale University, and earned a B.S. in Microbiology and Immunology from the University of Miami (Florida).



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



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