MarketVue®

Paroxysmal Nocturnal Hemoglobinuria (PNH)

July 2022

REACH
Market Research

MarketVue®: Paroxysmal Nocturnal Hemoglobinuria

UNDERSTAND THE PNH MARKET

MarketVue market landscape reports combine primary (KOL interviews and survey data) 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 (4 U.S. Hematologists, 1 UK Hematologist, 1 Italian Hematologist, 1 French Hematologist, and 1 German Hematologist), a quantitative survey with 23 U.S. physicians and secondary research.

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

- Apellis Pharmaceuticals
- Hoffmann-La Roche
- Novartis
- Regeneron
- Alexion / AstraZeneca

- Amgen
- Biocryst
- Arrowhead

DRUGS MENTIONED

- Ravulizumab (Ultomiris)
- Eculizumab (Soliris)
- Pegcetacoplan (Empaveli)
- Crovalimab (PiaSky)
- Iptacopan / LNP023 (Fabhalta)
- Pozelimab (Veopoz)

- Cemdisiran / ALN-CC5
- Danicopan / ALXN2040 (Voydeya)
- ABP-959 / Eculizumab biosimilar
- Vemircopan / ALXN2050
- BCX-9930
- Aro-C3

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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

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.