

MarketVue®

Chronic Refractory Gout

February 2024



MarketVue®: Chronic Refractory Gout

UNDERSTAND THE CHRONIC REFRACTORY GOUT 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 6 qualitative interviews with key opinion leaders (U.S. Rheumatologists), a quantitative survey with 20 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



MarketVue®: Chronic Refractory Gout

UNDERSTAND THE CHRONIC REFRACTORY GOUT MARKET

COMPANIES MENTIONED

- LG Chem
- Jiangsu Hengrui Pharmaceuticals
- JW Group / Simcere
- Selecta / Cartesian Therapeutics
- Sobi Biosciences
- Teijin America
- InventisBio
- ArthroSi Therapeutics
- Shanton Pharma
- Atom Bioscience
- Horizon Therapeutics / Amgen
- Nippon Chemiphar
- Protalix BioTherapeutics
- Arrowhead Pharmaceuticals
- Hinoa Pharmaceuticals

DRUGS MENTIONED

- Colchicine (Colcris, Mitigare)
- Allopurinol (Zyloprim)
- Febuxostat (Uloric)
- Probenecid (Probalan)
- Pegloticase (Krystexxa)
- Tigulixostat / LC350189
- Ruzinurad / SHR4640
- Epaminurad / URC102
- SEL-212
- TMX-049
- D-0120
- AR882
- SAP-001
- ABP-671
- HZN-003
- HZN-007
- NC-2500
- PRX-115
- HZN-457
- HP501

MarketVue®: Chronic Refractory Gout

Table of Contents

1. DISEASE OVERVIEW	4 - 5
A treatment-resistant inflammatory arthritis causing flares of severe joint pain and inflammation	4
Figure 1.1. Gout pathophysiology	4
Numerous risk factors for severe gout, but few truly refractory cases	5
Table 1.1 Gout risk factors	5
2. EPIDEMIOLOGY & PATIENT POPULATIONS	6 - 7
Disease Definition	6
Figure 2.1. G6 diagnosed prevalent cases of refractory gout by region	6
Table 2.1 Diagnosed prevalent populations of gout and refractory gout in the U.S. and EU5	6
Annualized incidence	7
Figure 2.2. Amgen/Horizon uncontrolled gout epidemiology funnel for U.S.	7
Table 2.2. Diagnosed annualized incident cases of gout and refractory gout in the U.S. and EU5	7
3. DIAGNOSIS & CURRENT TREATMENT	8 - 15
Overview	8
Figure 3.1. Referral and diagnostic pathway for gout patients	8
The vast majority of patients achieve flare control with available treatment options	9
Table 3.1. Treatment goals for chronic refractory gout	9
Table 3.2. Types of urate lowering therapies	9
Treatment flow for gout	10
Figure 3.2. Treatment algorithm for gout	10
Krystexxa (pegloticase)	11
Figure 3.3. Krystexxa safety and efficacy w/ and w/o methotrexate	11
Physician perspectives on Krystexxa	12
Figure 3.4. Pegloticase – upsides and downsides	12
Figure 3.5 Surveyed rheumatologist-estimated Krystexxa share and discontinuation rate	12
Physician perspectives on Krystexxa (cont)	13
Key treatment dynamics that will shape disease management and drug use in gout	14
Table 3.3 Must-know treatment dynamics in chronic refractory gout	14
Improved uricase and uricosuric formulations may vastly change the gout treatment landscape	15
Figure 3.6. Important dynamics of chronic refractory gout market evolution	15

MarketVue®: Chronic Refractory Gout

Table of Contents

4. UNMET NEED	16 - 17
Overview	16
Figure 4.1. Top unmet needs in chronic refractory gout	16
Physician perspectives on unmet needs in chronic refractory gout	17
5. PIPELINE ANALYSIS	18 - 20
Overview	18
Table 5.1. Urate lowering therapies in late-stage development	18
Results from Sobi and Selecta's Phase III trial of SEL-212 uricase therapy rivals Krystexxa with methotrexate	19
Figure 5.1. SEL-212 Phase 3 safety and efficacy	19
Uricosuric agents dominate the late-stage pipeline, but there are novel uricases in early development	20
Table 5.2. Urate lowering therapies in early-stage development	20
6. VALUE & ACCESS	21 - 23
Overview	21
Table 6.1. Current pricing of gout therapies, U.S.	21
Table 6.2. Typical U.S. commercial payer prior authorization criteria for Krystexxa	21
Payer financial burden of chronic refractory gout	22
Figure 6.1. Mean annual healthcare costs per patient by healthcare resource for refractory gout	22
Horizon has developed a number of programs to increase access and affordability of Krystexxa	23
7. METHODOLOGY	24 - 25
Primary Market Research Approach	24
Epidemiology methodology	25

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.