Optimizing Access for Orphan and Rare Disease Drugs

New treatments for rare diseases are rapidly entering the drug development pipeline and market, but there are substantial challenges in bringing orphan and rare disease drug treatments to market and to patients who need them.

Evidera offers an integrated approach and suite of services to clients to help solve these challenges, including developing a value demonstration plan, generating the necessary evidence outlined in the plan, and persuasively communicating the value evidence to payers and other key audiences.

Evidera's Approach to Orphan Drug Development Challenges

Challenge	Evidera Approach and Expertise
Determining number of patients affected worldwide	Defining market size using methods that range from literature reviews to chart reviews and database studies, and employing appropriate statistical methodology
Understanding patient characteristics and disease natural history, including presentation, disease progression, and relevant clinical outcomes	Designing epidemiologic studies utilizing a variety of data sources and applying appropriate statistical methodology and modeling to project disease course. When retrospective data are not enough, we can supplement them with prospective data collection
Designing and conducting robust, well controlled randomized safety and efficacy studies (particularly challenging due to the rarity of these diseases)	Providing creative solutions to demonstrate long-term outcomes, such as identifying and integrating data from multiple sources including observational studies, registries, and patient advocacy groups to project the disease course and potential impacts of a new therapy
Identifying the best market access route for our particular drug given the evolving process across countries to get orphan and rare drugs to market	Developing strategies for orphan drugs across a range of conditions and tailoring those strategies to key markets. The plans need to encompass not only evidence generation (and how these might be adapted from the approach for mainstream products) but also other market access and communication activities
Selecting the most appropriate clinical outcome assessments (COAs), which includes clinician-reported outcomes (ClinROs), patient-reported outcomes (PROs), observer-reported outcomes (ObsROs), and performance outcomes (PerfOs), to effectively capture outcomes important to patients with rare diseases. COAs may not exist or existing COAs may be insensitive to demonstrating treatment benefits	Developing and validating disease-specific novel COAs in rare disease populations to achieve regulatory approvals and inform health technology assessment (HTA) decision making

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Understanding what type of economic model will be required by commissioners	Developing a range of methodologically sound economic models for various commissioners based on need
Identifying and selecting HTA methods that sufficiently take into account the value that society places on access to therapies for patients with rare diseases – e.g., conventional methods may not account for society's willingness to pay more to help patients with rare, severe, life-limiting/threatening, and often genetic conditions	Understanding and implementing methods, such as selection of endpoints meaningful to patients and multi-criteria decision analysis (MCDA), that are designed to demonstrate value across a broader range of factors
Communicating the value of rare disease treatments to healthcare decision makers who may have limited awareness of the clinical, humanistic, and economic burden of rare diseases	Understanding that real-world evidence, such as registries, chart reviews, patient/caregiver surveys, and early economic models, can help to demonstrate the true burden of an under-recognized illness, and then tailoring the methods to meet specific challenges Our specialized market access writers can develop dossiers, slide decks, formulary kits, and monographs to educate healthcare decision makers on the burden of illness and unmet need in rare diseases. Evidera scientists have helped to shape the guidelines, including contributing to the 2013 update of the AMCP dossier format pertaining to specialty pharmaceuticals
Implementing post-marketing authorization studies to demonstrate real-world safety and efficacy when required	Designing innovative epidemiology studies involving large-scale databases, prospective data collection, post-launch safety study design and analysis

Selected Publications and Presentations in Orphan and Rare Diseases

Benjamin K, **Vernon MK**, Patrick DL, Perfetto E, Nestler-Parr S, Burke L. Patient-Reported Outcome and Observer-Reported Outcome Assessment in Rare Disease Clinical Trials: An ISPOR COA Emerging Good Practices Task Force Report. Value Health. 2017 Jul - Aug;20(7):838-855. doi: 10.1016/j.jval.2017.05.015.

Vernon MK, **Raluy-Callado M**, Trundell D, et al. Development of a Measure to Assess Severity of MPS II: The Disease Severity Score. Poster session presented at the ISPOR 18th Annual European Congress; November 2015; Milan, Italy.

Wyrwich KW, Auguste P, **Yu R**, Zhang C, Dewees B, Winslow B, Yu S, Merilainen M, Prasad S. Evaluation of Neuropsychiatric Function in Phenylketonuria: Psychometric Properties of the ADHD Rating Scale-IV and Adult ADHD Self-Report Scale Inattention Subscale in Phenylketonuria. Value Health. 2015 Jun;18(4):404-12.

Wiklund I, **Raluy-Callado M**, Chen WH, Muenzer J, Fang J, Whiteman D. The Hunter Syndrome – Functional Outcomes for Clinical Understanding Scale (HS-FOCUS) Questionnaire: Item Reduction and Further Validation. Qual Life Res. 2014 Nov; 23(9):2457-62.

Revicki DA, Brandenburg NA, Muus P, **Yu R**, Knight R, Fenaux P. Health-related Quality of Life Outcomes of Lenalidomide in Transfusion-dependent Patients with Low- or Intermediate-1-risk Myelodysplastic Syndromes with a Chromosome 5q Deletion: Results from a Randomized Clinical Trial. Leuk Res. 2013 Mar; 37(3):259-265.