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Reimbursement dynamics and market access hurdles for orphan drugs in the EU5

As the debate surrounding rising drug prices and healthcare costs intensifies, the high cost of orphan drugs has come under scrutiny. Approvals of orphan drugs, which are specifically developed to treat rare diseases, have grown dramatically in recent years. As a result of these approvals and the high prices associated with many orphan drugs, the treatment of rare diseases is an increasing burden to healthcare systems. While orphan drug development is supported by commercial and regulatory incentives, those drugs that have recently garnered EMA approval in rare indications have met with mixed success in HTA assessments across the EU5. In some cases, such as Vertex Pharmaceuticals' Orkambi for cystic fibrosis in the United Kingdom and France and PTC Therapeutics' Translarna for DMD in Germany, HTA reviews were negative because of the lack of compelling efficacy data and price negotiations failed on the grounds of cost-effectiveness, thereby precluding meaningful uptake of these drugs among patients who lack approved options. Orphan drug developers must integrate lessons from pioneering orphan drugs in the EU5 and effectively balance pricing expectations with clinical profile and uptake potential to optimize market access. On the other hand, EU5 healthcare agencies must find ways to manage their ever-tightening budgets in order to best serve the areas of high unmet medical need that include rare and orphan diseases. Our research, based on a survey conducted with 300 physicians across the EU5 specializing in various orphan diseases, including cystic fibrosis, idiopathic pulmonary fibrosis, Duchenne muscular dystrophy, spinal muscular atrophy, immune thrombocytopenic purpura, and beta thalassemia, analyzes the evolving dynamics affecting market access and reimbursement of current and emerging orphan drugs.

Biography

Akash Saini PhD is a Lead Analyst with the Infectious, Niche, and Rare Diseases team at Decision Resources Group, where he specializes in a diverse group of rare diseases. He received his PhD in biochemistry and biotechnology from the International Centre for Genetic Engineering and Biotechnology (ICGEB), New Delhi, India and his M.Sc. in biotechnology from Jawaharlal Nehru University, New Delhi, India. Prior to joining Decision Resources Group, Akash was a postdoctoral fellow at the University of Massachusetts Medical School, where he studied mitochondrial dysfunction in amyotrophic lateral sclerosis (ALS).

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