MARKET REPORT

Frontier Pharma: Duchenne Muscular Dystrophy and Becker Muscular Dystrophy - Identifying and Commercializing First-in-Class Innovation

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Summary

Highly Innovative and Diverse Pipeline

The Duchenne Muscular Dystrophy (DMD) and Becker Muscular Dystrophy (BMD) pipeline consists of 84 molecules across all stages of development. GBI Research's analysis revealed a high degree of innovation and diversity in this indication, with 70% of the pipeline being first-in-class products, acting on 13 first-in-class targets. This exceptional first-in-class innovation is largely due to the high number of first-in-class products solely targeting the dystrophin gene, which is the primary genetic cause of DMD and BMD. The strong presence of first-in-class products in the pipeline therefore creates a distinctly different landscape to the market landscape, which relies on symptomatic treatment glucocorticoids. Although Translarna (ataluren) is developed to correct the genetic defects, significant unmet needs remain in the market, as the treatment is applicable to only 10–15% of all DMD cases caused by nonsense mutations.

Despite a strong focus on personalized treatments that treat the genetic cause of the disease in the DMD/BMD pipeline, innovation is also concentrated on novel molecular targets that alleviate the dystrophic pathology regardless of gene mutations, thereby allowing widespread use in contrast to the mutation-specific treatments. These therapies are expected to be used alongside primary treatment to repair the mutated gene, halt muscle degeneration, and improve life expectancy of patients in the future market.

Strong Alignment of Innovation to Genetics and Disease Processes in Early Pipeline

DMD, and BMD, which is the less severe form, are neuromuscular diseases caused by heritable mutations in the single dystrophin gene, which ultimately lead to progressive muscle weakness and degeneration due to destabilization of the sarcolemma (muscle cell membrane) and the resultant loss of muscle integrity. However, increasing evidence suggests that multiple secondary pathological mechanisms, rather than dystrophin deficiency alone, cause or contribute to the pathological features of DMD/BMD and drive disease progression. This further substantiates the need for better understanding of the downstream events of dystrophin deficiency to enable the identification of more potential molecular targets that in turn could be translated into disease-modifying treatments.

Our proprietary analyses show that the 13 first-in-class targets differ substantially in terms of clinical and commercial potential based on how well their functional roles align to the disease pathophysiology and the strength of evidence in Preclinical studies. Some molecular targets are therefore considered more promising than others due to a stronger potential to be translated into novel treatments. The most promising targets provide a strong scientific rationale to support their therapeutic development, as indicated by substantial improvement in both muscle histopathology and function in vivo across different animal model systems.

Analysis also indicates opportunities for some of the first-in-class DMD/BMD targets to be repositioned to other MDs, although this is expected to be challenging given the currently limited understanding of the common

molecular processes defected across multiple types of MD.

Numerous Investment Opportunities in Deals Landscape

Strategic consolidation is relatively uncommon in the DMD/BMD market, with 15 licensing agreements and 18 co-development deals between 2006 and April 2015. Supported by findings from the industry-wide analysis, there is a tendency for first-in-class DMD programs to attract higher deal values than non-first-in-class programs, thus highlighting their commercial attractiveness. Despite the high-risk profile of first-in-class products, they have greater potential to revolutionize or improve therapeutic options, meaning that identifying promising first-in-class compounds early in development offers the greatest potential commercial benefit to pharmaceutical companies.

With 36 first-in-class products that are currently in development having not yet been involved in a licensing or co-development deal, there are numerous opportunities for in-licensing or co-development in this indication

Scope

The report analyzes innovation in DMD/BMD in the context of the overall pipeline and current market landscape. In addition, it analyzes the deals landscape surrounding first-in-class products in DMD/BMD and pinpoints opportunities for in-licensing.

The report covers and includes -

- A brief introduction to DMD/BMD, including symptoms, pathophysiology, and an overview of pharmacotherapy and treatment algorithms

- The changing molecular target landscape between market and pipeline and particular focal points of innovation in the pipeline

- A comprehensive review of the pipeline for first-in-class therapies, analyzed on the basis of stage of development, molecule type, and molecular target

- Identification and assessment of first-in-class molecular targets, with a particular focus on early-stage programs for which clinical utility has yet to be evaluated, as well as literature reviews of novel molecular targets

- Assessment of the licensing and co-development deal landscape for DMD/BMD therapies and benchmarking of deals involving first-in-class versus non-first-in-class-products

Reasons to buy

The report will assist business development and enable marketing executives to strategize their product launches, by allowing them to -

- Understand the focal shifts in molecular targets in the DMD/BMD pipeline

- Understand the distribution of pipeline programs by phase of development, molecule type, and molecular target

- Access scientific and clinical analysis of first-in-class developmental programs for DMD/BMD, benchmarked against non-first-in-class targets

- Access a list of the first-in-class therapies potentially open to deal-making opportunities

Additional Details

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