Above mentioned table presents the key metrics for Duchenne muscular dystrophy (DMD) in the six major pharmaceutical markets (6MM) (US, France, Germany, Italy, Spain, and UK) covered in this report during the forecast period from 2014–2019.

**DMD Market Will Witness Intense Growth between 2014 and 2019**

GlobalData estimates the 2014 sales for DMD reached approximately $8.2m across the 6MM, primarily driven by the sale of generic corticosteroids. By the forecast end in 2019, GlobalData expects a staggering growth in sales to $990.0m, at a Compound Annual Growth Rate (CAGR) of 160.5% across the 6MM over the five-year timeframe. GlobalData expects the US and the five European Union markets (5EU) (France, Germany, Italy, Spain, and UK) to contribute 44.8% and 55.2%, respectively, to the total market in 2019, corresponding roughly to the diagnosed prevalent cases in each region of 6,048 cases in the US compared with 11,612 cases across the 5EU in 2014. At the same, GlobalData also expects pipeline therapies in the US to be priced at a premium of approximately 10–30%.

Major drivers of growth in the DMD market during the forecast period are attributed to:

- The introduction of novel, high-priced, disease-modifying therapies for DMD, such as PTC Therapeutics’ Translarna, BioMarin/Prosensa Therapeutics’ drisapersen, and Sarepta Therapeutics’ eteplirsen.
Executive Summary

- The strong demand from the patient community for an effective therapy for this fatal disease and a strong positive influence from patient advocacy groups on drug development, regulatory approval processes, and ultimately, drug uptake.
- The expected approval of repurposed molecules with broad specificity to treat DMD, such as Santhera Pharmaceutical’s Catena (idebenone) and Eli Lilly’s tadalafil.
- The label expansion of exon-51 skipping therapies (specifically, BioMarin/Prosensa Therapeutics’ drisapersen and Sarepta Therapeutics’ eteplirsen) to non-ambulant patients will substantially increase the size of the target patient pool for these drugs.

Major barriers to the growth of the DMD market during the forecast period are attributed to:

- The applicability of disease-modifying therapies is limited to specific DMD mutations and therefore, only a small segment of the DMD patient population.
- The high cost of novel disease-modifying drugs may prevent their reimbursement by local health authorities and health insurance companies.
- The lack of standardization for measuring clinical efficacy across all stages of DMD. Currently, most studies for DMD use the change in the six-minute walk test (6MWT) as the primary clinical endpoint, which applies only to ambulant patients and excludes children under the age of five as well as non-ambulant patients.
- Clinical trials for label expansion to non-ambulant patients started in late 2014, but data are not yet available.

Below mentioned figure outlines the sales forecast for DMD in the US and 5EU from 2014 to 2019.

Sales for DMD by Region, 2014–2019

Mutation-Specific Therapies Emerge as the Major R&D Breakthrough and Future Market Drivers for DMD

DMD is caused by a variety of mutations in the dystrophin gene and targeting specific mutations is a strategy being used by drug companies in this space. PTC Therapeutics’ Translarna targets patients with nonsense mutation forms of DMD (nmDMD), whereas Sarepta Therapeutics’ eteplirsen and BioMarin/Prosensa Therapeutics’ drisapersen promote the bypassing of frameshift...
mutations and are commonly referred to as exon-51 skipping therapies. Mutation-specific therapies are being developed for a niche subgroup within the DMD population that has amenable mutations, and drug companies are expected to price these drugs at six figures per year for each patient. So far, Translarna, eteplirsen, and drisapersen are targeting ambulant patients with amenable mutations and the latter two drugs are in Phase III clinical trials. In August 2014, Translarna was granted conditional approval by the European Medicines Agency (EMA) based on Phase II efficacy data, prior to completion of its Phase III trials, which are currently underway and are expected to complete in June 2015.

GlobalData believes that Translarna and the exon-51 skipping therapies – drisapersen and eteplirsen – will be the primary drivers of growth in the DMD market. The mutation-specific drugs are expected to have a high price point, owing to their novelty, efficacy, and orphan drug status. Together, the mutation specific therapies are expected to contribute 85.6% to the DMD market across the 6MM in 2019 (see Figure: Sales for DMD by Pipeline Drugs in 2019). In particular, given their high demand and high attainable price, the exon-51 skipping therapies – drisapersen and eteplirsen – are expected to be a hot spot for growth in the DMD market.

Below mentioned figure outlines the global sales forecast for key DMD pipeline drugs in 2019.

### Sales for DMD by Pipeline Drugs in 2019

<table>
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<th>Drug</th>
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<tr>
<td>Eteplirsen</td>
<td>$189.3m</td>
</tr>
<tr>
<td>Drisapersen</td>
<td>$213.9m</td>
</tr>
<tr>
<td>Catena</td>
<td>$110.2m</td>
</tr>
<tr>
<td>Corticosteroids</td>
<td>$7.3m</td>
</tr>
<tr>
<td>Tadalafil</td>
<td>$24.8m</td>
</tr>
</tbody>
</table>

Source: GlobalData

Below mentioned figure illustrates GlobalData’s competitive assessment for the late-stage DMD pipeline products compared with the current standard of care, corticosteroids, during 2014–2019.

### Competitive Assessment of Key Pipeline Drugs for DMD, 2014–2019

- Translarna
- Eteplirsen
- Catena
- Drisapersen
- Tadalafil

---

**Commercial Score**

- Translarna: 3.00
- Eteplirsen: 3.00
- Catena: 3.00
- Drisapersen: 3.00
- Tadalafil: 2.75

**Clinical Score**

- Standard of care: 3.75
- Corticosteroids: 4.00

Source: GlobalData
Executive Summary

High Unmet Need for an Effective Disease-Modifying Therapy Remains in the DMD Market

There is a large unmet need for therapies to treat the DMD patient population who are not amenable to the mutation-specific therapies currently in the late-stage pipeline, namely, PTC Therapeutics’ Translarna, Sarepta Therapeutics' eteplirsen, and BioMarin/Prosensa’s drisapersen. Among the DMD patient population, 74.2% are ineligible for the aforementioned novel mutation-specific therapies. The unaddressed patient population is expected to benefit somewhat from repurposed pipeline drugs, Santhera Pharmaceutical’s Catena and Eli Lilly’s tadalafil, as these drugs are expected to treat specific stages of DMD progression, irrespective of the type of DMD mutation. Catena’s target indication is DMD patients with declining pulmonary function, whereas tadalafil, commonly available under the brand name Cialis for erectile dysfunction (ED) and benign prostatic hyperplasia, is expected to treat ambulatory patients ages five years and older. That being said, the patients with unaddressed DMD mutations are yet to receive disease-modifying therapies comparable to the mutation-specific drugs discussed above. Furthermore, DMD patients who have been overlooked by drug developers to date are newborns and young infants with DMD as well as late-stage DMD patients who are non-ambulant. There are no standardized protocols for measuring clinical efficacy across all stages of DMD, especially for the newborn and wheelchair-bound patient population. Overall, in spite of the rapid developments in DMD therapy over the last five years, there still remains a large untapped DMD patient population who would benefit from effective disease-modifying therapies. This signifies opportunity for current and future drug developers to tap into.

Exon-Skipping Platform May Increase Target Patient Population

Sarepta Therapeutics’ eteplirsen and BioMarin/Prosensa Therapeutics’ drisapersen are developed from proprietary antisense oligonucleotide (AON) platform chemistries, which can be used to target several other mutations in addition to exon-51. These additional mutation-specific molecules are termed as “follow-on” antisense oligonucleotide exon-skipping therapies (follow-on AONs). Theoretically, up to 83.0% of all DMD mutations can be targeted via follow-on exon-skipping AON therapy. As of now, BioMarin/Prosensa and Sarepta have six and seven early-stage (preclinical and Phase I) follow-on AON molecules, respectively, which hold the potential to target up to an additional 22.0% of DMD patients. Furthermore, expansion of the drisapersen and eteplirsen drug labels to include non-ambulant patients has immense clinical and commercial potential, as this could greatly increase their target patient pool by approximately two-fold. However, key opinion leaders (KOLs) interviewed by GlobalData suggested that clinical trial data would be necessary for label expansion. Sarepta
Executive Summary

was first to initiate clinical trials to expand eteplirsen’s label to treat non-ambulant DMD patients. BioMarin/Prosensa Therapeutics is also expected to pursue a similar pathway to increase the opportunity for their exon-51 drug, drisapersen. Overall, developers of exon-skipping therapy have a high potential to increase their target patient population using existing drug development platforms, without the need for discovering entirely novel strategies.

What Do Physicians Think?

KOLs interviewed by GlobalData highlighted that the environment surrounding DMD pipeline therapies is encouraging and drug development in this area is active.

“Definitely, there is a big momentum right now [for] Duchenne and a lot of work that [still] needs to be done, and it needs to be done quickly… to really move things forward. It’s a good time for Duchenne. It’s never been this busy before, I can tell you.”

US Key Opinion Leader

Experts have a highly positive opinion of novel disease-modifying therapies in the late-stage pipeline.

“Both drugs [eteplirsen and drisapersen] show very promising effect[iveness]. All of the studies go in the same direction, so it seem[s] very promising. There’s one study with effects for eteplirsen in only 10 patients, but it is also very promising. Those patients [in the study] appear to be very stable [over the trial period]. In both stud[ies], we need a little bit more data, but it seems that both of them are very promising.”

EU Key Opinion Leader

“I think that all patients with a nonsense mutation will receive the drug [Translarna], as long as the Social Security System accepts to pay for it.”

EU Key Opinion Leader

There appears to be a strong desire from KOLs interviewed by GlobalData to expand the emerging novel therapies, such as Translarna and exon-51 skipping drugs, to the broader non-ambulant DMD patient population.

“The main issue, for me, is that [mutation-specific therapies] only come through for ambulant patients, and that two thirds of the patients are non-ambulant. So, for most approaches, we don’t have a single clue if it’s efficient or not for non-ambulant patients. For me, that’s one of the main concerns.”

EU Key Opinion Leader
Executive Summary

There is strong opinion among KOLs around newborn screening and the possibility of introducing treatment at an earlier age for diagnosed DMD patients.

“I think it’s going to be critical to have newborn screening done on a nationwide basis and hopefully, a worldwide basis, as the key to treatment effectiveness may in part be related to the time of onset, [and] the initiation of these treatments. Then it makes sense that the earlier treatment is initiated, the greater the possibility of long term – and significant – therapeutic benefit would then follow.”

US Key Opinion Leader

Due to the high anticipated price of some novel disease-modifying drugs, KOLs stressed that payer reimbursement, especially considering the potential for expanded patient populations, will be an important factor for drug availability.

“There will be very difficult decisions to make, ‘Are we going to prescribe this drug, which I’m sure will be expensive?’ Also, ‘Should I prescribe the drug to the non-ambulatory [patients] with stop codon [mutations]?’ My inclination will be ‘Yes,’ because we don’t have anything better, but I hope the insurance [companies] will agree. That’s the issue.”

US Key Opinion Leader
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Introduction

2 Introduction

2.1 Catalyst

The global Duchenne muscular dystrophy (DMD) market is currently served by generic corticosteroids and lacks dedicated therapies for treating DMD. The strong demand and lack of efficacious therapies specific for DMD will allow several pipeline products to emerge uncontested. The nascent DMD market, estimated at $8.2m in 2014, is expected to change radically with the launch of disease-modifying pipeline therapies. The following catalysts have spurred significant attention on the global DMD market:

- On August 4, 2014, PTC Therapeutics’ Translarna was granted conditional approval in the European Union (EU) based on Phase IIb studies. This marks the first approval of a disease-modifying therapy for treating DMD patients who have nonsense mutations. The conditional approval also draws attention towards the niches within the DMD market and the opportunity for drug developers to target niche DMD patient segments.

- Exon-51 skipping therapies, drisapersen and eteplirsen, have demonstrated promising clinical efficacy in improving walking abilities during Phase II trials, and are likely to receive regulatory approval by 2016. Such anticipated drug approvals would add two premium-priced orphan drugs, greatly increasing the DMD market size.

- Strong demand for effective disease-modifying therapies is at an all-time high from patients, advocacy groups, and clinicians. Regulatory agencies such as the US Food and Drug Administration (FDA) have begun acknowledging this unmet need and are incorporating strategies such as expedited drug approvals to address this need, and have included the DMD community in the drug development process.

2.2 Related Reports


Introduction

Appendix

10.7 About GlobalData

GlobalData is a leading global provider of business intelligence in the healthcare industry. GlobalData provides its clients with up-to-date information and analysis on the latest developments in drug research, disease analysis, and clinical research and development. Our integrated business intelligence solutions include a range of interactive online databases, analytical tools, reports, and forecasts. Our analysis is supported by a 24/7 client support and analyst team.

GlobalData has offices in New York, San Francisco, Boston, London, India, Korea, Japan, Singapore and Australia.

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