Table below presents the key metrics for celiac disease in the six major pharmaceutical markets (6MM) (US, France, Germany, Italy, Spain, and UK) covered in this report during the forecast period from 2013–2018.

### Key Metrics in Six Major Pharmaceutical Markets for Celiac Disease, 2013–2018

#### 2013 Epidemiology

<table>
<thead>
<tr>
<th>Metric</th>
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<tbody>
<tr>
<td>Prevalent population (≥15 years; 6MM)</td>
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<tr>
<td>Diagnosed population (≥15 years; 6MM)</td>
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#### 2013 Market Sales

<table>
<thead>
<tr>
<th>Region</th>
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<tr>
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<tr>
<td>5EU</td>
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<tr>
<td>Total</td>
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#### Pipeline Assessment

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</tr>
<tr>
<td>Number of first-in-class drugs</td>
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#### Key Events (2013–2018)

- Larazotide acetate launch in the US, expected in Q1 2018 (Level of Impact: ↑↑↑)
- Latiglutenase launch in the US, expected in Q1 2019 (Level of Impact: ↑↑↑)
- Larazotide acetate launch in the 5EU, expected in Q1 2019 (Level of Impact: ↑↑↑)
- Latiglutenase launch in the 5EU, expected in Q1 2020 (Level of Impact: ↑↑↑)

#### 2018 Market Sales

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<td>Total</td>
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</tr>
</tbody>
</table>

Source: GlobalData

---

Table below presents the key metrics for celiac disease in the 6MM covered in this report during the forecast period from 2013–2023.

### Key Metrics in Six Major Pharmaceutical Markets for Celiac Disease, 2013–2023

#### 2013 Epidemiology

<table>
<thead>
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<th>Metric</th>
<th>Value</th>
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<td>Prevalent population (≥15 years; 6MM)</td>
<td>4.8 million</td>
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#### Key Events (2013–2023)

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- Latiglutenase launch in the US, expected in Q1 2019 (Level of Impact: ↑↑↑)
- Larazotide acetate launch in the 5EU, expected in Q1 2019 (Level of Impact: ↑↑↑)
- Latiglutenase launch in the 5EU, expected in Q1 2020 (Level of Impact: ↑↑↑)

#### 2023 Market Sales

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Source: GlobalData

5EU = France, Germany, Italy, Spain, and UK; 6MM = US and 5EU
Executive Summary

The Celiac Disease Market Will More than Triple between 2013–2018 and Grow over Ten Times by 2023, with the US Contributing 99% Market Share

In 2013, the base year of this forecast, GlobalData estimates the celiac disease market size was $49.6m across the 6MM. Sales in the base year were made up of sales from generic medicines used to manage refractory or severe forms of the disease. The US contributed 99% of these sales, generating an estimated $49.4m. This is due to the use of highly priced pharmaceuticals and a significantly higher diagnosed prevalence of celiac disease in the US market.

By the end of the five-year forecast period in 2018, celiac disease sales are forecast to grow to $162.2m at a Compound Annual Growth Rate (CAGR) of 26.77% over the five-year period. The 5EU markets will experience slower growth than the US market as drug launches are expected to occur outside of the five-year forecast in the 5EU.

A key event during the five-year forecast in this report is the anticipated introduction of Alba/Teva’s larazotide acetate pipeline therapy in the celiac disease market. Larazotide acetate is being developed for use as an adjunctive treatment to a gluten-free diet (GFD), which is the current standard of care for celiac disease. Larazotide acetate is forecast to launch in the US at the end of the five-year forecast period, in Q1 2018. A key competitive advantage of larazotide acetate is that it is expected to target the majority of the disease population compared with the 4–7% of the refractory population that are currently treated with generics including steroids and immunosuppressants.

To account for the market dynamics expected to occur in the celiac disease space over the latter end of the next decade, in this report, GlobalData has incorporated a 10-year forecast case study to showcase the potential impact that key pipeline drug launches in the 5EU and US will have on the market. In Q1 2019, larazotide acetate is expected to launch in the 5EU and Alvine/AbbVie’s latiglutinase, also a GFD adjunctive treatment, may potentially launch in the US. These pipeline drugs are forecast to grow the sales in the 6MM to $289.9m. The following year, Q1 2020 will see the launch of latiglutinase in the 5EU, thereby boosting the 6MM sales further to $343.3m, with a steady incline up to $551.1m by 2023 at a CAGR of 27.24% over the 10-year time frame. GlobalData anticipates that latiglutinase could potentially be prescribed in combination with larazotide acetate, leading to similar uptake and sales of the two therapies. Meanwhile, larazotide acetate may raise awareness of celiac disease and upcoming pharmacological interventions, leading to a higher year-on-year growth for latiglutinase.
Executive Summary

Major drivers for the growth of the celiac disease market over the forecast period include:

- The introduction of two novel pipeline drugs, Alba/Teva’s larazotide acetate and Alvine/AbbVie’s latiglutenase, which will be used as an adjunct to the current standard of care: a GFD.
- The combined use and high uptake of larazotide acetate and latiglutenase.
- The growing diagnosed celiac disease population.

Major barriers to the growth of the celiac disease market include:

- Phase III efficacy and safety data for celiac disease pipeline products are unknown and products could fail in late-stage development.
- The high cost of gluten-free foods makes following a GFD expensive, therefore, it is likely that the prices of adjunctive treatments will be discounted or launched at a low price.
- Lack of physician and patient awareness along with a lack of confidence in pipeline drugs in comparison with a GFD. In the 6MM, increased physician education, coupled with increased cost-consciousness, would support better disease management using the standard of care and would reduce the level of opportunity for novel agents.

- Developments in the gluten-free food market may further refine the GFD and this, in turn, may deter physicians from prescribing adjunctive treatments.

Figure below illustrates celiac disease sales for the 6MM during the five-year forecast period from 2013–2018.
Executive Summary

Figure below illustrates celiac disease sales for the 6MM during the 10-year forecast period from 2013–2023.

Drug Developers Are Trying to Identify Novel Therapeutic Targets by Exploring Licensing Opportunities and the Formation of New Partnerships

The current standard of care for celiac disease is non-pharmacological and involves following a GFD where gluten is excluded entirely from a patient's diet. A GFD, although potentially quite expensive due to highly priced gluten-free foods, is generally very successful. However, a significant proportion of patients are unable to completely exclude gluten and thus, either suffer from inadvertent gluten exposure or have problems with compliance. Therefore, one of the greatest unmet needs in disease treatment is to completely exclude gluten from celiac patients' diets. In order to address this need, companies have focused their research toward adjunctive therapies that would support a GFD to exclude all gluten from celiac sufferers.

There is also a trend toward small biotechnology companies actively pursuing out-licensing opportunities for their celiac disease drugs, and alliances are being arranged by a wide spectrum of pharmaceutical and biotechnology companies. This is because the vast majority of novel therapeutic agents for celiac disease are being developed by small pharmaceutical and biotechnology companies. For example, larazotide acetate and latiglutenase were initially developed by small biotechnology companies, Alba and AbbVie, respectively, which have subsequently collaborated with the much larger pharmaceutical companies, Teva and AbbVie, respectively. Larger pharmaceutical companies are readily investing time and money into biotechnology companies that are focusing on celiac disease drug development, but companies such as GlaxoSmithKline (GSK), via GSK-Avalon, are also attempting to delve into the celiac disease market at the preclinical level.

Due to celiac disease being an indication that has only started to generate interest in recent years, research and development (R&D) is focused largely on identifying novel targets in the celiac
disease mechanism and developing drugs to prevent the onset or development of the disease. A greater knowledge of the celiac disease immunopathological pathway has led to the identification of various points where drugs can prevent disease progression. Many of the therapeutic interventions being developed serve as an adjunct to GFD, but there is a focus on disease-modifying drugs and drugs for niche patient groups, such as refractory celiac disease (RCD). GlobalData anticipates that licensing activity and the formation of new partnerships, coupled with new innovative therapies, will steer smaller companies to continue researching novel compounds with a consistent stream of funding coming from larger pharmaceutical companies that are trying to invest in the celiac disease market.

Diagnosis of Celiac Disease, Awareness and Adherence to a Gluten-Free Diet Are the Greatest Unmet Needs in the Celiac Disease Market

A GFD is usually an effective treatment of celiac disease, thus leading to improvements in clinical symptoms. However, the diagnosis of celiac disease is a convoluted and drawn out process that sometimes, physicians have relatively little knowledge about. As such, the celiac disease market suffers from low rates of diagnosis, giving rise to a need for more refined methods of diagnosis.

Although there is a need for approved products, a GFD can usually prevent disease progression in the majority of cases. For this reason, Key Opinion Leaders (KOLs) interviewed by GlobalData (during May to September, 2014) have highlighted that there is a need for improvements in physician education and patient awareness of the GFD, which should result in fewer referrals to specialist gastroenterologists.

A significant unmet need in celiac disease lies in the lack of available products in the market to treat the indication. There are currently four drugs in the celiac disease clinical-stage pipeline and all of these products have a novel mechanism of action (MOA). Three of the four drugs in the clinical stages of development are designed to be used as adjunctive treatments to a GFD. After successful Phase IIb trials were reported in July 2014, the most advanced late-stage drug is Alba/Teva’s larazotide acetate, which is a tight junction modulator preventing absorption of gluten in the small bowel. Alvine/AbbVie’s latiglutenase is an enzyme designed to break down gluten peptides into non-toxic forms and is expected to complete Phase IIb trials in Q1 2015. Meanwhile, BioLineRx’s BL-7010 has completed Phase I/II trials and is a synthetic polymer designed to bind to gluten peptides in the small bowel, allowing them to be excreted. NexVax-2 represents the fourth and earliest stage drug in Phase I development. NexVax-2 is being developed by ImmusanT and works as a vaccine to prevent reactions to gluten in
Executive Summary

celiac patients who have a human leukocyte antigen (HLA)-DQ2 phenotype. NexVax-2 could potentially be a game-changer, completely removing the need for a GFD.

Due to the existence of gluten in most foods, adhering to a GFD can prove to be a difficult task for some patients with the non-refractory form of the disease. However, compliance is enhanced by the growing gluten-free food industry, which allows patients to consume foods that would otherwise contain gluten, but now contain a gluten substitute instead. Nonetheless, these foods are expensive and are poorly palatable. Therefore, a clinical unmet need exists for patients on a GFD who either need assistance with the exclusion of all gluten from their diets with adjunctive therapies, or who need a disease-modifying treatment because they cannot comply with a GFD.

Novel Gluten-Free Diet Adjunctive Pipeline Agents Are Expected to Target a Large Proportion of Celiac Patients Suffering from Gluten Exposure

The celiac disease pipeline has progressed over the last few years and the two most advanced drugs are in Phase IIb stage of development. After successful Phase IIb trials were reported in July 2014, the most advanced late-stage drug is Alba/Teva’s larazotide acetate, which is a receptor blocker that modulates tight junction proteins to reduce gluten absorption through the small intestine. Alvine/AbbVie’s latiglutenase is an enzyme designed to break down gluten peptides into non-toxic forms and is expected to complete Phase IIb trials in Q1 2015.

The clinical-stage pipeline targets a larger population of non-refractory celiac patients (approximately 95% of the entire celiac disease population) and those without severe complications of the disease. Patients can suffer from gluten exposure either from inadvertent intake or failure to comply with a GFD. This means that the pipeline drugs are not directly competing for patients with severe injury or refractory patients who are currently using steroids and immunosuppressants. That said, the development of Alba/Teva’s larazotide acetate and Alvine/AbbVie’s latiglutenase could indirectly affect patients with the severe forms of injury or the refractory patient population by preventing the initial occurrence of gluten-induced injury.

The KOLs interviewed by GlobalData revealed that both larazotide acetate and latiglutenase would act as a preventative treatment, and would be used as a supplement to a GFD. Both drugs are likely to complement each other as they target different parts of the disease mechanism; thus, they are likely to be prescribed early on and in combination. This would mean that there would be less intense competition between the two drugs after their launch. However, latiglutenase currently has no definitive efficacy data as it has not completed Phase IIb trials, which will end in Q1 2015. For this reason, GlobalData’s competitive assessment of the celiac disease landscape places larazotide...
Executive Summary

Acetate as the more clinically attractive pipeline agent when compared with latiglutensase. A competitive assessment of the celiac disease pipeline therapies is provided in Figure below.

What Do Physicians Think?

The KOLs interviewed by GlobalData highlighted that while novel therapies are certainly needed to target the celiac disease population, a greater unmet need in celiac disease is for improved physician education and patient awareness in order to improve diagnosis.

“…there are many, many patients who are undiagnosed today. There are many problems. The main problem I’ve seen is the interest of doctors to diagnose these patients. Many of these patients are undiagnosed because the doctors who see these patients don’t [understand] the symptoms of the disease. The problem is difficult to solve, because many doctors need to better [understand] the symptoms of celiac disease. Education [is needed for] practitioners; [general practitioners] mainly.”

Key Opinion Leader, September 2014

The KOLs also indicated that there is a need for novel drugs in the celiac disease market.

“I think there will be a very rapid uptake of these medications by the community; the celiac community has been very attentive to this field for a long time. It’s one of the most common questions I get when somebody’s either newly diagnosed or even in follow-up: When is there going to be a pill? How is the pill coming along? I think these developments are eagerly awaited by the patient population, and I think when something is approved, an awful lot of people are going to want it right away.”

Key Opinion Leader, May 2014

“…I think GFD adjunctive therapies are something that almost everyone with celiac disease will have, if [they are] safe and effective, [patients] will have [them] in their medicine cabinet.”

Key Opinion Leader, May 2014
Executive Summary

“Maybe in the near future, [the] discovery of new drugs and new actions will be very welcome to these [celiac] patients.”

Key Opinion Leader, September 2014

The KOLs also discussed the novel GFD adjunctive therapies, larazotide acetate and latiglutenase, which GlobalData anticipates will launch during the next ten years.

“I think it’s [larazotide acetate] exciting. I think the mechanism of action is really interesting.”

Key Opinion Leader, May 2014

“I do think this [larazotide acetate] is going to be [used] along with the gluten-free diet. It’ll make you not have to be as careful and it’ll give you the same or better disease control with less effort, basically.”

Key Opinion Leader, May 2014

“It [larazotide acetate] is a very promising product and has a good safety profile.”

Key Opinion Leader, September 2014

“I think it [latiglutenase] would be used very much in the same way [as larazotide acetate]. I think they are competing medications with probably a similar patient population but very different mechanisms of action. [They] may be differently useful in different patient populations and may actually be useful in conjunction in some patients, as well.”

Key Opinion Leader, May 2014

“…I think it’s a very sensible dual therapy [larazotide acetate and latiglutenase] because it’s attacking the same pathway at two different points. [Therefore,] I think everyone wants to be first on the market, but beyond that, I think there’s plenty of room for both therapies and I think it’ll raise awareness so much, that I think actually there’d still be synergy to having more than one on the market.”

Key Opinion Leader, May 2014

“Latiglutenase contains enzymes to protect cells from gluten and specifically [digests] glutamine and proline, and it’s also a very attractive way of treating these patients. [Latiglutenase works in the] intestinal lumen [where it] digests all [gluten-derived] proteins, so it will be a very promising route [of therapy, and will therefore, seem attractive to celiac] patients. [Latiglutenase] [has yet] to be studied in Phase IIb studies and I have an excellent opinion about this product.”

Key Opinion Leader, September 2014
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2 Introduction

2.1 Catalyst

The global celiac disease market is very naive with no disease-specific drugs available, although it does have a growing early-stage clinical and preclinical pipeline. This leaves a great need and opportunity for pharmacological intervention. GlobalData expects this market to undergo substantial growth between 2013 and 2018, and then further still, through 2023, growing by more than 10 times over the 10-year period. The main drivers of this large expansion will be: the launch of novel pipeline drugs that target a majority of the celiac disease population, a high uptake of novel drugs, and increasing cases of diagnosed celiac disease patients.

The standard of care for celiac disease is a gluten-free diet (GFD) without any pharmacological drugs specifically against celiac disease. The market only includes inexpensive generic steroids and immunosuppressants that target those with severe reactions to gluten or with a refractory form of the disease, which is approximately 5% of the population. Following the product launches in the mid-to late-term of the forecast period, the pipeline drugs are set to target patients on a GFD that undergo gluten exposure due to either an inadvertent intake, or a failure to comply to a GFD. Despite the fact that the GFD is highly effective, there is an increasing population of patients for whom a GFD is problematic and difficult to follow.

As such, the need for pharmacological treatment options for patients who are unable to afford expensive gluten-free alternatives, or for patients with other obstacles that leave them unable to comply with their GFD has been recognized. The pharmaceutical industry is responding and exploring a number of new therapies that offer hope to these patients. Two novel pharmacological treatments are Alba/Teva's larazotide acetate and Alvine/AbbVie's latiglutenase. These have the potential to be used as adjunctive treatments to a GFD. In addition, these drugs will most likely be used in combination and will potentially help contribute to the effectiveness of a GFD. The development of these drugs targets a larger patient population, so they may seem attractive to drug developers.

In all, the lack of pharmacological drugs in celiac disease presents an exciting opportunity for the entry of novel GFD adjunctive and disease-modifying agents for refractory and non-refractory celiac disease (non-RCD) treatment.
Introduction

2.2 Related Reports


2.3 Upcoming Related Reports

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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