Executive Summary

Table below presents the key metrics for acromegaly and gigantism 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.

### Acromegaly and Gigantism: Key Metrics in the 6MM, 2013–2018

<table>
<thead>
<tr>
<th>Year</th>
<th>Epidemiology</th>
<th>Market Sales</th>
<th>Pipeline Assessment</th>
<th>Most Promising Pipeline Drugs</th>
<th>Key Events (2013–2018)</th>
<th>2018 Epidemiology</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>2013</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>2013</td>
</tr>
<tr>
<td></td>
<td>Acromegaly and gigantism, diagnosed prevalent population</td>
<td>25,945</td>
<td></td>
<td></td>
<td></td>
<td>25,945</td>
</tr>
<tr>
<td></td>
<td>2013</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>2013</td>
</tr>
<tr>
<td></td>
<td>US</td>
<td>$382m</td>
<td></td>
<td>Novartis' Signifor LAR (pasireotide)</td>
<td>Patent expiry of Novartis' Sandostatin LAR Depot (octreotide acetate for injectable suspension) in the US in 2014</td>
<td>Acromegaly and gigantism, diagnosed prevalent population</td>
</tr>
<tr>
<td></td>
<td>5EU</td>
<td>$206m</td>
<td></td>
<td>Chiasma's oral octreotide (octreotide acetate)</td>
<td>Launch of Novartis' Signifor LAR in the US and 5EU in 2015</td>
<td>26,743</td>
</tr>
<tr>
<td></td>
<td>Total</td>
<td>$588m</td>
<td></td>
<td></td>
<td>Patent expiry of Ipsen's Somatuline Depot (lanreotide) Injection in the 5EU in 2015</td>
<td></td>
</tr>
</tbody>
</table>

### 2018 Market Sales

<table>
<thead>
<tr>
<th>Market</th>
<th>Sales</th>
</tr>
</thead>
<tbody>
<tr>
<td>US</td>
<td>$478m</td>
</tr>
<tr>
<td>5EU</td>
<td>$229m</td>
</tr>
<tr>
<td>Total</td>
<td>$707m</td>
</tr>
</tbody>
</table>

**Source:** GlobalData

*Oral octreotide is considered a first-in-class drug as it is the first ever oral formulation of octreotide. ATL1103 is the other first-in-class drug, since it is the first antisense drug to be developed in this market.

5EU = France, Germany, Italy, Spain, and UK; 6MM = US and 5EU; LAR = long-acting repeatable

### Acromegaly and Gigantism Market Will Have Moderate Growth between 2013 and 2018

GlobalData estimates the 2013 sales for acromegaly and gigantism at approximately $588m across the 6MM covered in this report. The US contributed to 65% of these sales, generating an estimated $382m. This is nearly double that of the market for the five European Union countries (5EU) (France, Germany, Italy, Spain, and UK) and can be attributed mainly to the higher prices of pharmaceuticals in the US, and the slightly higher prevalence of the disease in the US.

By the end of the forecast period in 2018, acromegaly and gigantism sales are forecast to grow to $707m at a Compound Annual Growth Rate (CAGR) of 3.74% over the five-year period. The forecast period will be marked by the patent expirations of all the currently marketed drugs; however, this will not have a major impact on branded drug sales due to the lack of generic competition during the forecast years. The US market is expected to grow faster than the 5EU one, at a CAGR of 4.58%, reaching sales of $478m in 2018. The higher price pressures in the
Executive Summary

European region have led to the lower pricing of pharmaceutical drugs, and hence, the overall sales figures in the 5EU will be much lower than in the US. The 2013 base-year sales for acromegaly and gigantism in the 5EU were $206m; GlobalData expects this market to grow at a CAGR of 2.10% to reach sales of $229m in 2018.

Major drivers for the growth of the acromegaly and gigantism market over the forecast period will include:

- The increased use of existing and pipeline somatostatin analogs (SSAs) with better administration or dosing regimens, due to the recent focus on improving patient convenience in this chronic disease market.
- The increased use of existing agents, such as Somavert, and pipeline agents, such as Signifor LAR, for the treatment of the refractory patient population, which was recently expanded due to the discovery of patients resistant to current SSAs.
- Recent trends in the 5EU showing an increased use of SSAs in place of surgery as a first-line treatment.

Major barriers to the growth of the acromegaly and gigantism market will include:

- The high cost of the drugs and long-term treatment regimens, which hinder the use of pharmacologic agents in this market and places surgery, the cheaper one-time effective option for many patients, as the first-line therapy across the 6MM.
- Low disease awareness among patients and physicians, which remains a crucial unmet need in this field and leads to a lower disease diagnosis rate, a delayed diagnosis, and a lack of comorbidity management.
- The sparsely populated late-stage pipeline, which will hinder the growth of this market during the forecast years.
- The dominating presence of Novartis’ Sandostatin franchise for the last 25 years in the field, which makes it difficult for new entrants, especially from the same drug class, to establish themselves in the market.
**Executive Summary**

Figure below outlines the sales forecast for acromegaly and gigantism in the US and 5EU from 2013 to 2018.

**Increased Focus on Patient Convenience Leads to a Trend towards Better Drug Delivery Methods**

The acromegaly and gigantism market is dominated by injectables, including all the currently marketed and branded drugs. Due to the chronic nature of the diseases and the reliance on long-term pharmacological therapy, increased importance is being given for developing easier administration routes and for reducing the treatment burden of patients. This trend is clearly visible in the research and development (R&D) strategies employed by companies in this area and also in the selection of current pipeline drugs. One of the major thrusts in innovation among the pipeline drugs is the development of easy-to-use formulations of existing octreotides. This is evident from the octreotides that are currently being developed by Chiasma and Camurus. Camurus’ drug, octreotide FC, which is licensed and being developed by Novartis, is based on a ready-to-use liquid crystal depot formulation of octreotide chloride to be injected subcutaneously once a month. Since this drug can be self-administered, it removes the inconvenience of having to go to a health administrator every month.

Chiasma’s oral octreotide, based on a novel transient permeability enhancer (TPE) drug delivery technology, will be the first oral formulation of an SSA, thus providing an easy-to-use, needle-free option for patients. Glide Pharma’s Glide octreotide, which will be moving soon into clinical development, is also based on an innovative delivery system. It consists of a solid dose needle-free drug application system that is reusable, simple to use, and can be self-administered by the patient at home.

**High Unmet Need Remains for Developing Novel Drugs to Treat Refractory Patients and Managing Disease Comorbidities**

The SSA drug class is the most commonly used pharmacological treatment for acromegaly and gigantism patients, and it is effective in around 60% of the population. However, the discovery of an increasing number of patients who display resistance to this drug class and other existing treatments calls for exploring other novel
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mechanisms of action. Refractory patients, comprising about 20–30% of the total patient population, are urgently in need of alternative treatments and present a major unmet need in this disease market. Pharmaceutical companies are currently trying to address this need by mostly focusing on SSAs with better efficacy and safety profiles. However, according to GlobalData’s interviews with Key Opinion Leaders (KOLs), such approaches will not fulfil this major unmet need in the market as the potential of the SSA class has been exhausted and has reached the limit of its efficacy. There remains the need for a drug that simultaneously offers tumor control, biochemical control, and symptom control, which will not be fulfilled by the currently marketed drugs or the pipeline drugs during the forecast period. Investigating the molecular aspects of the growth hormone (GH) receptor can help unravel new drug targets, while exploring chimeric or multi-ligand drugs provides other promising avenues for research.

Another highly unfulfilled unmet need, which has been unanimously emphasized by KOLs, is the control of comorbidities associated with the disease. Patients are often diagnosed with multiple conditions like heart disease, diabetes, sleep apnea, or arthropathy, in addition to the disease itself, and these comorbidities are the major reasons for increased mortality in patients. Treating patients with comorbidities is highly challenging, and the physician has to develop individualized treatment strategies suited to each patient’s specific clinical conditions, making acromegaly and gigantism very difficult to treat. The focus of existing medications has been more towards achieving biochemical and tumor control and less towards addressing these accompanying complications. As a result, acromegaly-specific medications that successfully provide biochemical remission fail in curing the lingering comorbid conditions of the patient. Despite recent advances in increasing comorbidity awareness among physicians, as exemplified by the publication of the Acromegaly Consensus Group’s (ACG’s) treatment guidelines, there remains the need for developing specific combination therapies targeted towards acromegaly and gigantism and their associated comorbidities. Overall, these unmet needs present multiple opportunities for new and existing pharmaceutical developers to gain a competitive edge over existing players and to provide a better treatment experience for patients.

Patient and Physician Education Is Crucial to Reduce Delay in Diagnosis in Acromegaly

Acromegaly and gigantism are rare diseases occurring in only a small section of the population; therefore, low disease awareness among patients and physicians is a natural consequence. Additionally, acromegaly is also characterized by an insidious onset which delays diagnosis even further, whereas gigantism is characterized by a dramatic acceleration of growth and is detected more easily. Early-stage disease
Acromegaly and Gigantism – Opportunity Analysis and Forecast to 2018

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Characteristics for acromegalics are somewhat non-specific, and go unrecognized by the patient. Additionally, initial assessments are usually conducted by primary care physicians having inadequate experience with the disease; some may not have ever seen such a patient in their practice. As a result, when patients are finally confirmed with a diagnosis of acromegaly, they are years into the disease and ridden with multifarious comorbidities, which complicates treatment significantly. An early diagnosis could lead to an increased likelihood of a cure by the standard-of-care treatments for this disease. Therefore, patient and physician education are very crucial for reducing the delay in diagnosis and improving treatment outcomes in case of acromegaly. Furthermore, since patients are often diagnosed with acromegaly while seeking medical attention for orthopedic, dental, rheumatologic, or cardiac disorders, such education should include physicians of these various specialties. Patients also require to be educated so that they can manage their comorbidities better.

Sparse Pipeline Will Limit Market Growth – Sandostatin LAR Will Remain Market Leader

The sparse pipeline will limit the growth of the acromegaly and gigantism market over the forecast period from 2013–2018. There are only three drugs in late-stage development and GlobalData anticipates two of these to be launched in the next couple of years. With the approval of one of them, Novartis’ Signifor LAR, physicians will have more options to tackle the difficult-to-treat acromegaly patients who are resistant to current treatments. The launch of the second drug, Chiasma’s oral octreotide, will provide patients with an option to avoid painful injections with the easy-to-use oral alternative. The third drug, Antisense Therapeutics’ ATL1103, is an antisense therapy also targeted to treat refractory patients. However, after assessing all three drugs against standard-of-care therapies, GlobalData believes that the pipeline agents do not match up to the current treatments. Furthermore, in spite of the rising shares of Pfizer’s Somavert and Ipsen’s Somatuline Depot, Sandostatin LAR Depot will maintain the lion’s share in this disease market at the end of the forecast period.

While Signifor LAR demonstrated greater efficacy than the standard of care in treating refractory acromegaly patients in clinical studies, it is also riddled with a higher chance of hyperglycemia adverse events. Since diabetes is a common comorbidity occurring with acromegaly, KOLs have raised a red flag about the use of Signifor LAR. Due to these safety concerns, GlobalData has assigned a lower clinical score to Signifor LAR compared with that of Sandostatin LAR Depot, as illustrated in Figure below. The commercial score of Signifor LAR was also slightly lower than that of Sandostatin LAR Depot due to the size of its target patient pool, which will exclude diabetic and pre-diabetic patients. The assumed higher price of Signifor LAR also contributed negatively to its...
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commercial score, as this will impede reimbursement and drug usage, especially in the 5EU. Considering KOL viewpoints, GlobalData expects that the uptake of Signifor LAR will be slow, and physicians will exercise caution in prescribing the drug, only reserving it for a small segment of patients. The results from the Phase IV trial of Signifor LAR which is expected to be completed by 2017, if positive, can boost the sales of the drug and increase its chances of being considered as first-line drug therapy.

In the case of oral octreotide, the active ingredient is the same tried-and-tested octreotide drug that has been used successfully for many years in acromegaly. Therefore, physicians indicated that they would be comfortable in prescribing the drug and that many patients were looking forward to the oral formulation. However, factors like meal-spacing issues and a high dosing requirement of the drug lowered its clinical score with respect to that of Sandostatin LAR Depot. In addition, the main impediment for the uptake of the oral drug is its commercial profile. Chiasma Pharma, which is developing the drug, has no commercialization experience in acromegaly and no presence in the 6MM, which attributes to the poor commercial score of the drug. GlobalData expects that oral octreotide will have a slow uptake, considering that it has to compete with Sandostatin’s 25 years of franchise experience in this market.

ATL1103, which recently completed Phase II trials, scored almost as high as Somavert on the clinical scale. However, ATL1103 lost out on its commercial score, since Antisense Therapeutics also has no marketing or commercialization experience. Moreover, GlobalData does not believe that the pipeline drug will be able to capture much of Somavert’s shares during the forecast period, considering its expected launch in late 2018 in the US. Added to that, is the fact that antisense therapy is still in an embryonic stage in the pharmaceutical armamentarium; thus, physicians will initially be wary about replacing Somavert with ATL1103.

Overall, despite its patent expiry in 2014, Sandostatin LAR Depot will continue to be the market leader at the end of the forecast period, due to the lack of generic competition; however, some of Sandostatin LAR Depot’s shares will be captured by the pipeline SSAs and Ipsen’s Somatuline Depot. Sales for Somatuline Depot have been growing in the US and 5EU in recent years, and GlobalData projects this trend to continue during the forecast years. The other currently marketed drug, Pfizer’s Somavert, is also anticipated to have a steady rise in shares, according to the positive feedback received for the drug by GlobalData’s KOLs.
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What Do the Physicians Think?

The KOLs interviewed for this report highlighted the need to explore newer mechanisms of action in order to develop novel drugs with better clinical profiles.

“I think we still lack a drug that simultaneously offers tumor control, biochemical control, and symptom control, and those tend to go hand in hand.”

US Key Opinion Leader, May 2014

“I think right now, we do not have any kind of groundbreaking approach. It's the same thing we have been doing for the past 20 to 30 years. It's the same thing. And the issue is just kind of relative efficacy versus relative cost versus relative side effects in individual patients. For some patients, you just give them a shot of Somatuline, Sandostatin LAR Depot, and they are fine. [With] some patients, you struggle and struggle and struggle, and nothing happens.”

US Key Opinion Leader, May 2014

“Personally, I think that the somatostatin receptor is pretty much exhausted at this point. I don't know that there's a lot more to be gained by beating on the same door.”

US Key Opinion Leader, May 2014

KOLs also indicated the unmet need for patient and physician education to tackle the low disease awareness associated with acromegaly and gigantism. This would also facilitate an earlier
Executive Summary

disease diagnosis and better comorbidity management.

“The biggest issue is just recognition of the disease and referral to somebody who deals with the disease. I think that's the major roadblock.”

US Key Opinion Leader, May 2014

“The other unmet need is on the lingering effects of the disease. The comorbid conditions that accompany the disease are often not sufficiently improved with medical therapy. I think that there needs to be more of a focus on educating people who are treating patients with acromegaly in how to address comorbid conditions, and what the better or the best means of treating these things are. Things like hypertension, diabetes, arthritis, osteoporosis, or fractures.”

US Key Opinion Leader, May 2014

“Generally…all the patients don’t know this disease. When we present the diagnosis, they hear the name of this disease for the first time. And they have to understand that the treatment is of this disease and not the treatment of diabetes, hypertension, and sleep apnea – each [a] systemic complication. This is a problem of counseling with the patient. And then, of course, we have to decide the treatment, and it is not so easy to choose the best option.”

EU Key Opinion Leader, July 2014

The KOLs also showed a certain level of scepticism regarding the late-stage pipeline drugs in the market and expressed doubt that these would completely replace the current standard of care.

“I think it [Signifor LAR] may be useful in a subset of patients. I think that there may be patients who are not responsive to the predecessor somatostatin analogs, but maybe it might be a benefit to them. The only hesitation I have is with the diabetes side effect; that is something that would not make me choose that over one of the currently available somatostatin analogs.”

US Key Opinion Leader, May 2014

“Oral octreotide is a good drug, because it’s an oral administration. I think that probably we don’t have strong advantages with respect to long-acting somatostatin analogs. But [it] is a new option in some cases, [it] is useful for patients who have difficulty to take an injection, for instance, and sometimes, we have this patient. But, for the others, it is maybe a little less effective than long-acting octreotide.”

EU Key Opinion Leader, July 2014
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2 Introduction

2.1 Catalyst

The global acromegaly and gigantism market is dominated by a handful of branded drugs and is characterized by a sparsely populated late-stage clinical pipeline. The forecast period between 2013 and 2018 will be marked by the patent expirations of these branded drugs; however their sales will not be impacted by this because of the lack of generic competitors. Along with the launch of three pipeline agents, GlobalData expects the overall market to have moderate growth, at a Compound Annual Growth Rate (CAGR) of 3.74% over the five-year period. The main drivers of this expansion will be the extended use of the marketed and pipeline agents for the treatment of difficult-to-treat acromegaly patients; as well as the uptake of the existing and pipeline somatostatin analogs (SSAs) with more convenient administration and dosing regimens.

The first-line drug treatment for acromegaly and gigantism consists of the drugs belonging to the SSA class. Two branded players comprise this class: the current market leader, Novartis' Sandostatin LAR (long-acting repeatable) Depot (octreotide acetate for injectable suspension), whose franchise has dominated the market for more than two decades; and Ipsen's Somatuline Depot (lanreotide), whose shares have been rising steadily in recent years. SSAs are effective in around 60% of patients; Pfizer's Somavert (pegvisomant) is the go-to drug for first-line drug failures. However, Somavert fails to achieve disease control in some cases, and overall, around 20–30% of patients are in urgent need for other therapeutic options. Rising to this need, Novartis has developed the pipeline drug, Signifor LAR (pasireotide), which is a second-generation SSA that is expected to launch in 2015. Towards the end of the forecast period, Antisense Therapeutics will launch ATL1103, an antisense therapy positioned as a second-line alternative to refractory patients. Another pipeline drug, Chiasma’s oral octreotide (octreotide acetate) promises to relieve patients from the hassle of painful injections by virtue of its first-in-class oral formulation.

Overall, the dominance of Novartis’ Sandostatin LAR Depot franchise presents a stiff barrier to the entry of these drug therapies, especially in the case of the pipeline SSAs, considering that they do not have a visibly significant edge over Sandostatin LAR Depot. Therefore, GlobalData believes that these pipeline drugs will have a slow start, and sales will pick up towards the end of the forecast period as patients and physicians become more accustomed to the relative strengths of these treatments.
Introduction

2.2 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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