



# Maximizing the rare chance of launch success with orphan drugs

*A strategic approach to achieving launch excellence and success for rare-disease medicines*

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# Executive summary

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Biopharmaceutical companies with limited experience in rare-diseases are increasingly looking to launch rare-disease drugs. There is also growing interest from early-stage companies in using the rare-disease pathway as a step to becoming fully integrated biopharmaceutical companies. These companies may have developed expertise in research and development of rare-disease indications, but lack insight and experience in launching a drug with a rare-disease indication. For these companies, it is important to understand and invest in preparation to achieve launch success in the rare-disease market.

In preparing to launch a rare-disease medication, it is important to understand the needs in the therapy area and invest strategically so as to maximize available resources and impact on the unique rare-disease environment. This article provides a framework for identifying the needs in a rare-disease therapy area, as well as another to prioritize investment for launch excellence and success. A case study is then provided to show how a biopharmaceutical company has successfully met the needs of a rare-disease therapy area.

# 1. You have one chance to get a launch right

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The launch of a medicine is a complex operation to carry out, and a significant number of launches fail to meet their pre-sales expectations<sup>1</sup>. Furthermore, the quality of a drug's launch impacts its financial performance well beyond the initial launch phase<sup>2</sup>. Inability to assess and understand the needs and wants of the market is listed as one of the main reason for product-launch failures<sup>3</sup>. Having supported numerous pharmaceutical companies in their product launch preparations, we have identified an important approach to apply in unveiling insights on the needs in the rare-disease market.

## **The treatment pathway is the ideal framework for identifying existing needs in rare-diseases**

For common and well-understood diseases with existing treatment options, much of the healthcare infrastructure to support the successful launch of a medicine is in place, as many patient needs are already met to a large extent. With rare-diseases, the picture is completely different. In many cases, the biopharma company has to develop a deep understanding of the needs in the therapy area before identifying ways to build the infrastructure needed for launch success.

One way of understanding needs in a rare-disease area is to follow the patient treatment pathway and compare the state of affairs in a typical rare-disease area with what exists in a common-disease area. The information needed to conduct such as exercise can be readily gathered by interviewing key opinion leaders (KOLs), healthcare professionals (HCPs) and patient advocacy groups (PAGs).

On a common disease patient treatment journey, patients transition from disease awareness and recognition to presentation and diagnosis by HCPs. Treatment options are then considered, a treatment is selected, and a brand best suited to the patient's needs is chosen (if there are multiple brands to choose from). The patient then follows an access pathway to secure the medicine, and takes it over a period of time (with

assistance or support if applicable) to achieve improvement in health outcomes.

## **Gaps along the rare-disease patient treatment pathway**

### **Firstly, rare-disease awareness**

Compared to common diseases, rare-diseases have very low awareness among members of the public and HCPs. This is because the incidence of the disease is very low, and therefore most people will not have heard of it. In addition, Primary Care Physicians get little or no training on these diseases. A lot of the information available on these diseases is provided by patients on blogs or internet sites that have not been medically vetted. Awareness creation for the disease falls mainly to PAGs.

### **Secondly, presentation and diagnosis**

The genetic variability of many rare-diseases results in highly variable presentations, which lead to increased difficulties in making the correct diagnosis. In some cases, several symptoms must be aggregated for a professional to come to a diagnosis of the rare-disease. Unfortunately, these symptoms may be presented to different specialists who may not suspect the presence of the rare-disease, and hence may not look for the other confirmatory symptoms to diagnose it. This can lead to a significant delay in receiving a correct diagnosis compared to timescales for diagnosing common diseases; in cases where a quick and accurate diagnosis is achieved, the patient often enters a period of intense effort to understand the condition and the treatment options. All of these can cause a lot of distress and suffering for the patient and their family members.

### **Thirdly, the treatment options**

In many cases, the treatment options for rare-diseases may be very few, with limited evidence to support selection and few specialists and treatment centers available to provide treatment. The treatment centers tend to be tertiary centers or academic

1 [http://www.pharmatimes.com/magazine/2016/january\\_2016/failure\\_to\\_launch](http://www.pharmatimes.com/magazine/2016/january_2016/failure_to_launch)

2 New-product success in the pharmaceutical industry: how many bites at the cherry? M. Corstjens, E. Demeire & I. Horowitz, Pages 319-331

3 <https://www.forbes.com/sites/steveolenski/2017/12/12/5-factors-that-can-make-or-break-your-marketing-strategy/#535c754c51e4>

medical centers; hence, they cover large geographies. This may mean significant travel for the patient to receive appropriate medical attention.

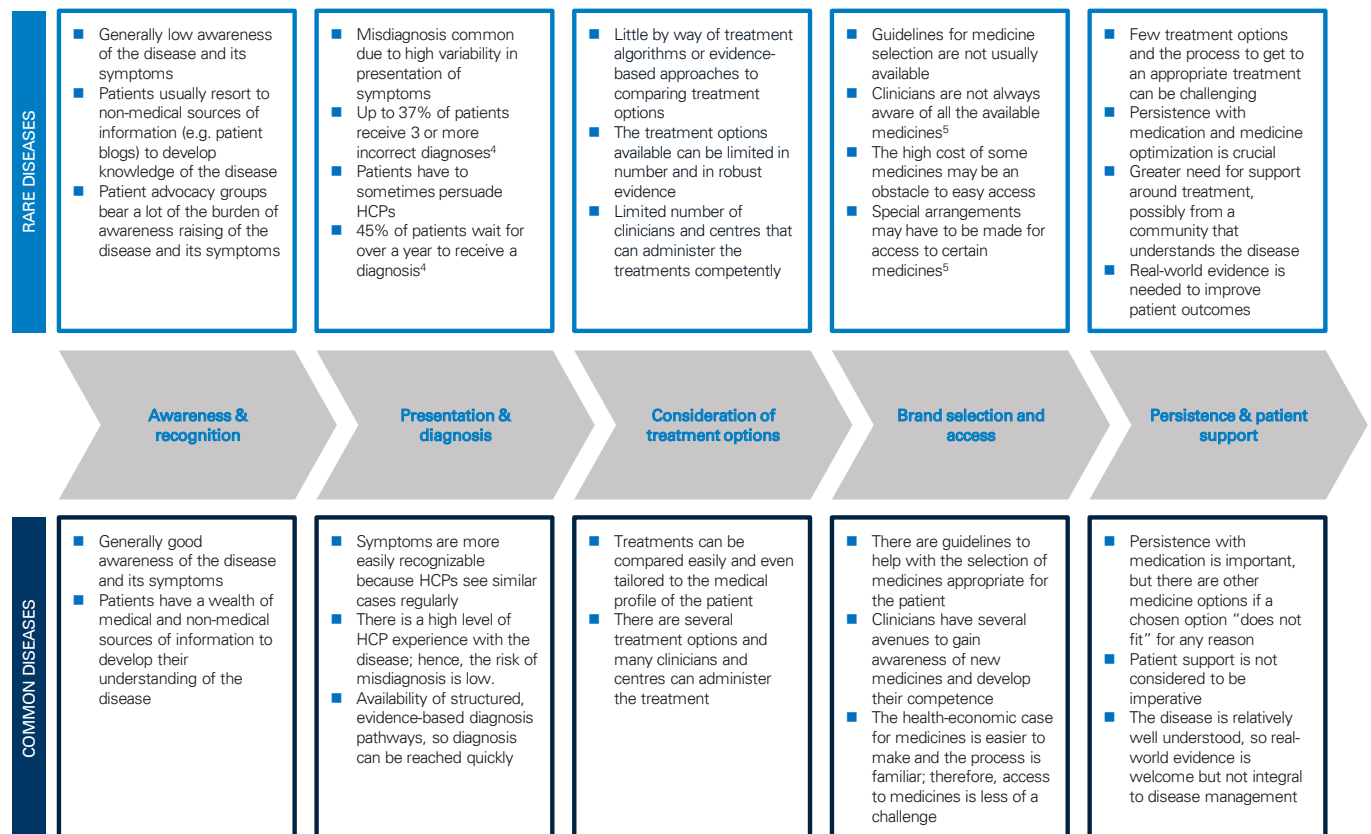
**Fourthly, access and brand selection**

Rare-disease medicines are usually expensive and for a relatively small number of people. It means access frameworks may need to be set up – in some cases on a patient-by-patient basis. In addition, reimbursement negotiation may be challenging, as the usual formula for calculating reimbursement may not apply. Furthermore, there may be no agreed guidelines or algorithms for brand selection, and therefore a wide variety of prescribing processes and treatment outcomes.

**Finally, persistence and patient support**

While persistence and patient support may not be necessary with common diseases, they are a big consideration for rare-diseases due to the low patient numbers and the significant medical issues patients and their caregivers confront. To maintain persistence, a number of patient-support provisions need to be proactively put in place. In addition, collection of real world evidence is usually a key feature in the study of rare-diseases, as the data collected can be used to gain insight, provide a resource for ongoing support systems and ultimately, be used to continuously improve patient outcomes.

Figure 1: Significant differences in patient experience (and needs) between rare and normal diseases



Source: Arthur D. Little

4 <https://www.rare-disease.org.uk/media/2995/activity-report-2016.pdf>  
 5 <https://ojrd.biomedcentral.com/track/pdf/10.1186/s13023-016-0401-7>

## 2. A successful launch begins by addressing needs around patient treatment

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Preparation for a product launch should begin up to three years in advance of the launch date, with several workstreams operating in parallel to ensure that an excellent launch is achieved. In addition to the usual pre-launch deliverables, rare-diseases have a number of extras that need to be in place for a successful launch.

### **Awareness and recognition**

It is imperative for companies to build strong relationships with the KOLs and PAGs in the rare-disease space and increase disease awareness. Unlike with common diseases, in rare-disease areas, the KOLs are few and may be scattered across a handful of treatment centers throughout a country or countries. Their support in creating awareness of the disease in the medical community, as well as receiving referrals from their colleagues, is imperative for success. PAGs play a great role in raising awareness and helping patients to recognize symptoms so they can guide their HCPs to the right diagnoses. It is also important to create a reputable source of information that patients and HCPs have a place to go to develop their basic understanding of the disease.

### **Presentation and diagnosis**

Patients with rare-diseases can receive multiple misdiagnoses before the right diagnosis is reached, which can cause untold suffering for them and their families. It is, therefore, an essential part of launch preparation to ensure that misdiagnosis is minimized and time from presentation to diagnosis is reduced. One way of doing this is to provide education to relevant HCPs. In addition, the relevant pathology tests to confirm diagnosis should be added to the usual battery of tests so the rare-disease is already in the frame of consciousness from an early stage. Furthermore, it is important to facilitate easy referral to tertiary centers where the KOLs are located, e.g., by creating a forum so HCPs can quickly discuss patient cases with KOLs online and make referrals or diagnoses as required.

### **Treatment options consideration**

For a number of rare-diseases, treatment options may include surgery, pharmacotherapy, treatment combinations, etc., with

little or no agreed guidance or information on how to decide which one to go for. For patients, it would be useful to have patient-friendly information to help them participate in the selection of appropriate treatments that covers direct treatments and supportive-care strategies. For HCPs, a consensus-driven algorithm based on the available evidence would be useful (if none already exists) in helping to decide treatment steps. Development of this algorithm can be facilitated by convening experts in the area to discuss and come to an agreed position for the benefit of the therapy area. Therefore, this becomes a critical deliverable for the launch-planning team.

### **Brand selection and access**

The reimbursement process for a rare-disease medication can be quite different from that of a common disease. The usual health-economic arguments are often more difficult to make due to smaller patient numbers, gaps in evidence, life-long therapy and complications that are not typical of common diseases. It means reimbursement discussions may need to be had on a retail rather than wholesale basis. In preparing for launch, it is important to have the infrastructure in place to pursue payment (and co-payments) on a case-by-case basis. In addition, prescribers have to be supported to secure funding for medication and be reassured that the selection of a brand will not attract additional work for them. Payers may also need to be seen as customers and supported in streamlining their processes to reduce the additional burden. Bridging infrastructure may need to be developed to unite patients with their medication – e.g., tests and diagnostics, hospital equipment, home-delivery, etc. – as these may not naturally be in place.

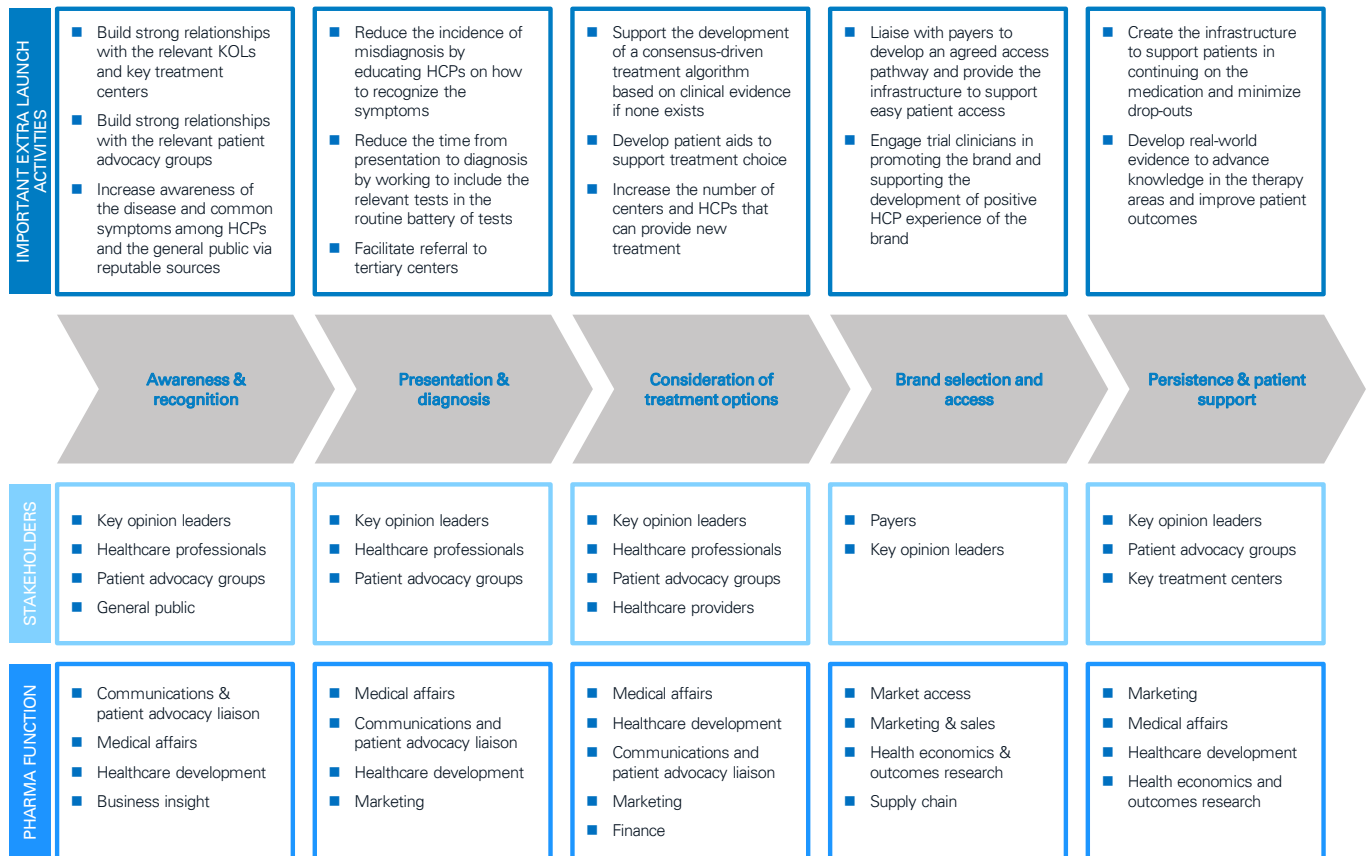
### **Persistence and patient support**

Patient numbers are very low in rare-diseases, and the process of getting them onto medications can be demanding for all involved. Therefore, it is in the best interest of all parties to ensure that the patient remains on the medication and gets the full benefit. To ensure persistence, it may be necessary to wrap the patient and caregiver with “beyond the pill” services such as psychological counseling, 24-7 nurse call-center support

and, home care. This helps to ensure that the full benefits of the medication are realized, the risk of drop-out is minimized, and the value of the medication is demonstrated. In addition, a patient toolkit can be put together to support the patient through

the treatment journey. It is also common for drug companies to support the creation of a registry (if none already exists). This ensures gathering of insight to support improvement in patient treatment and delivery of improved outcomes.

Figure 2: Important extra activities, stakeholders and relevant functions for a rare-disease drug launch



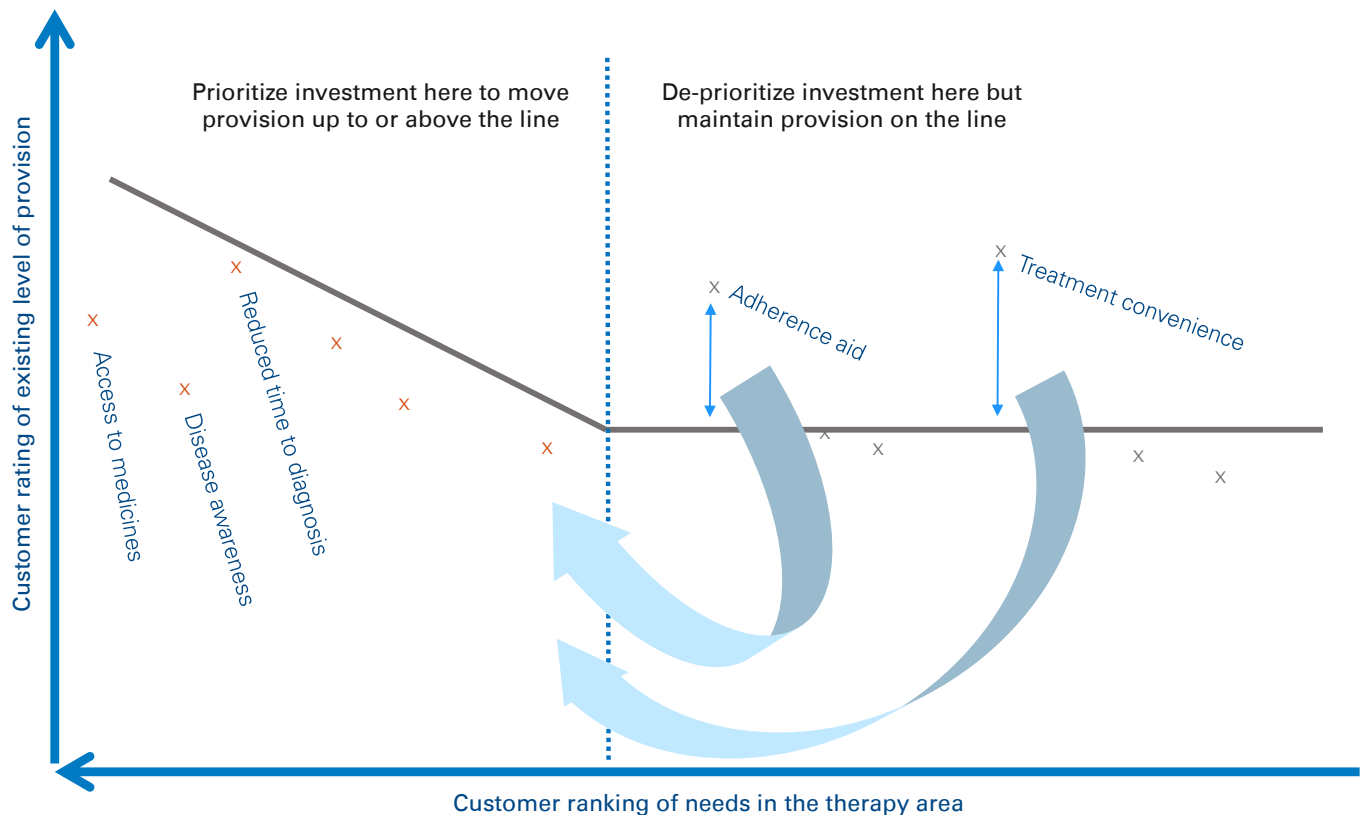
Source: Arthur D. Little

### 3. Launch investment can be strategically deployed for maximum impact

To maximize the impact of investment in launch activities, a “happy line” plot can be created from stakeholder feedback on needs in a therapy area and the existing level of provision. W. Chan Kim’s “Happy Line” theory<sup>6</sup> postulates that the chances of commercial success are relative to the degree to which companies address the most pressing needs of their customers – with a “Happy Line” that suggests the minimal target threshold of customer satisfaction. The happy line plot will give a picture of the current standard of care provision related to the minimum satisfaction levels of customers. This then shows where the gaps in provision are. Where resources are limited,

as is usually the case, priority should be given to those needs to the left (the slanting part) of the happy line. These are the needs considered the most pressing to a patient’s outcome measures. To achieve maximum impact, the most pressing needs should be addressed so that the level of customer satisfaction is reached or exceeded. For needs that are of less priority, the investment should simply meet the need up to the happy line. If a low-priority need is being met to a level significantly above the happy line, resources should be diverted from this activity to those with higher customer priority.

Figure 3: Illustrative “happy line” plot showing treatment needs and how to prioritize investment



Source: Adaptation of Chan Kim’s strategic canvas

6 <http://caseyzemanonline.com/wp-content/uploads/2011/05/BlueOceanStrategy.pdf> Happy line adapted from strategy canvas  
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## 4. Case study: Sanofi Genzyme's activities with Gaucher's disease

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Gaucher's disease is an inherited metabolic disorder in which a type of fat called glucocerebroside cannot be adequately degraded. This is because the body does not make enough of the enzyme glucocerebrosidase. The result is build-up of certain fatty substances in the liver, spleen, bone marrow and nervous system, which causes interference with normal functioning<sup>7</sup>. Disease prevalence is 1 in 100,000.

Sanofi Genzyme markets an Enzyme Replacement Therapy (ERT), - Cerezyme®, and a Substrate Reduction Therapy (SRT), - Cerdelga®, both for the treatment of Gaucher's disease (mainly the non-neuronopathic type). Sales performance of Cerezyme has been strong, although the launch of Cerdelga means patients can have an alternative, more convenient treatment. The medicine remains a cash cow for Sanofi Genzyme, with revenue of over \$800 million in 2017, which by far exceed analyst peak-sales predictions of \$300 million<sup>8</sup>.

To achieve success in the market, Sanofi Genzyme carried out a number of activities to meet needs along the patient treatment pathway.

### Awareness and recognition

Sanofi Genzyme partners with EURORDIS (a patient-driven alliance of patient organizations for rare-diseases) as well as the European Gaucher Alliance to raise awareness of the condition. In addition, evidence-based information is provided on a website for patients and HCPs<sup>9</sup>.

### Presentation and diagnosis

Diagnosing Gaucher's disease can be challenging, partly because other diseases share its symptoms. To enable speedy diagnosis, Sanofi Genzyme facilitated a convening of international experts to develop a consensus-based algorithm for diagnosis<sup>10</sup>.

### Treatment options consideration

ERT & SRT are the two main treatments for the non-neuronopathic type of Gaucher's disease. Sanofi Genzyme provides a website with information on the two classes of treatment. In addition, Sanofi Genzyme sponsored a gathering of international experts to develop a consensus-based algorithm for the management of patients after diagnosis<sup>11</sup>.

### Brand selection and access

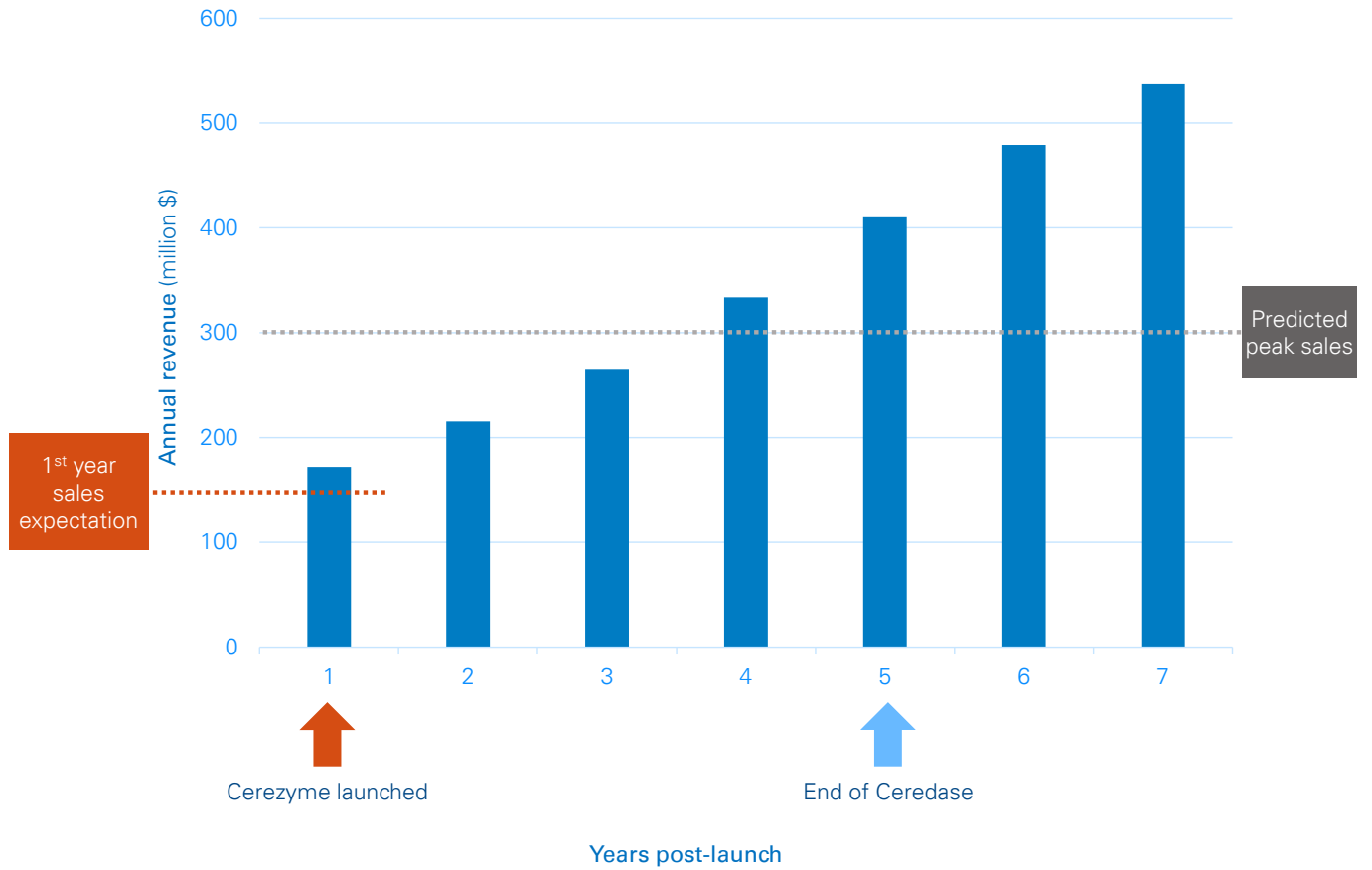
After the medication has been prescribed, Sanofi Genzyme case managers can help patients in a number of ways to get their medication. The services include support with treatment authorizations and appeals and financial assistance programs<sup>12</sup>.

### Persistence and patient support

Sanofi Genzyme provides a home-therapy service to support patients who have been stabilized on medication. This minimizes patient inconvenience and drop-out. Sanofi Genzyme also sponsors and manages a registry for Gaucher's disease<sup>13</sup>.

7 <https://www.gaucherdisease.org/about-gaucher-disease/what-is/>  
 8 [https://www.forbes.com/2003/05/30/cz\\_zm\\_0530genzyme.html#19cbe4694954](https://www.forbes.com/2003/05/30/cz_zm_0530genzyme.html#19cbe4694954)  
<https://www.nytimes.com/1994/05/25/business/company-news-new-enzyme-by-genzyme-is-approved.html>  
 9 <https://www.gaucher-disease.eu/en/hcp/disease-overview-0>  
 10 <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3058841/pdf/nihms238537.pdf>  
 11 <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3058841/pdf/nihms238537.pdf>  
 12 <https://www.sanofigenzyme.com/en/patient-support/patient-services/cerezyme-imiglucerase-for-injection-support/>  
 13 <https://www.registrynxt.com/Home/For-Patients-and-Families>

Figure 4: Genzyme achieved an excellent launch, with sales exceeding expectation in the launch phase and well into the product life cycle



Source: Genzyme annual report, ADL analysis

## 5. What kind of launch do you want for your rare-disease medicine?

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The launch of a rare-disease medicine presents a great opportunity to make a real difference to a community of patients, families and HCPs who are very keen to receive new solutions for the health challenges they face. The degree to which the solution meets the needs of the rare-disease community is related to the level of commercial success. Considering that a lot of resources are required to get the rare-disease drug through the regulatory approval process, it is only prudent to complete the last lap by investing in launch excellence and success.

As drug launches can take two to three years to prepare for, and rare-diseases usually get accelerated regulatory approval, it is important to start the launch preparations for a rare-disease medicine earlier in the drug development process. If you have a rare-disease medicine at the end of phase 2 clinical trials or in phase 3 clinical trials, the launch preparation process can begin, starting with gaining deep understanding of the treatment needs along the patient treatment pathway. This should then be followed by creation of a happy line plot to decide how investments can be made strategically to achieve launch success.

### **The ADL launch excellence team blends pharmaceutical product knowledge and local market understanding with product launch expertise.**

- We bring an industry-wide view on approaches taken at major pharmaceutical organizations in their launch readiness.
- We bring a senior, expert team who have experience in product launch assessment, planning, and implementation.
- We have supported over 190 launch planning and market assessment projects in the last eight years.
- We have supported and continue to support many rare disease companies to achieve commercial success.
- We understand the time and resource constraints associated with drug launches. Our team is ready to deploy immediately across most target markets.
- We collaborate. We have a collaborative approach to strategy development and ensure that the co-created knowledge is transferred fully to our clients.
- We use our experience and proven tools to enable launch success for our clients, we maintain a launch database with several thousand activities mapped.

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### Maximizing the rare chance of launch success with orphan drugs

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#### Arthur D. Little

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Our consultants have strong practical industry experience combined with excellent knowledge of key trends and dynamics. ADL is present in the most important business centers around the world. We are proud to serve most of the Fortune 1000 companies, in addition to other leading firms and public sector organizations.

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