In 2011, the global amyotrophic lateral sclerosis (ALS) market was estimated at $112m. The market is expected to decline at a negative Compound Annual Growth Rate (CAGR) of 5.7% and is estimated at $70m by 2019. The current treatment options are limited to Rilutek (riluzole) and off-label medications that offer symptomatic relief. Rilutek has been the only approved drug for ALS since 1995, and while it does extend survival, considerable unmet need still exists for a therapy that can inhibit disease progression significantly without serious side effects. The primary drivers of the market in the forecast period are an increasing population, especially among the elderly, as well as increasing disease awareness. The ALS pipeline has a few late-stage therapies that could potentially launch in the forecast period, such as ALS-02 (2015) and Radicut (edaravone) (in Japan only, 2013). While these products will expand the market, they will not transform the treatment paradigm. According to Key Opinion Leaders (KOLs), these new products will be mainly used as add-on treatments to Rilutek; KOLs believe a combination therapy would be most beneficial to patients due to the distinctive mechanisms of action of each drug. The primary barrier to market growth will be the anticipated patent expiry of Rilutek in 2013.
As the manufacturer of Rilutek, Sanofi has had a stronghold in the ALS market for over a decade now. Pipeline products, such as ALS-02 (2015), won’t enter the market until the latter half of the forecast period, if at all. Companies competing in the ALS market, including Biogen, Avicena, Mitsubishi Tanabe, and Sanofi, are all following the same strategy: to collaborate with or acquire smaller companies that have a well-established history of R&D in developing drugs for rare diseases.

Given the lack of disease-modifying treatment options and the orphan drug status of the disease (which provides numerous financial, marketing, and drug approval benefits), a huge market opportunity exists for companies that can develop an efficacious drug to treat and significantly improve the prognosis of the disease.

There are two first-in-class products in Phase III of development, namely, ALS-02 and Radicut. ALS-02 is an ultra-pure, clinical form of creatine, being developed by Avicena. Radicut (edaravone) is a free radical scavenger being developed by Mitsubishi Tanabe in Japan only. Healthcare professionals expect both of these products to be used as an add-on therapy to Rilutek. Therefore, the new products are expected to drive the ALS market in the forecast period but not transform the treatment paradigm or overcome revenue loss from Rilutek’s patent expiration. A recent setback for the ALS therapeutics pipeline has been the failure of dexpramipexole (KNS-760704), an orally administered drug in late-stage clinical development from Biogen.

ALS experts (neurologists and researchers alike) believe that lack of understanding of the pathophysiology and underlying etiology is a major barrier in the treatment of ALS. Various hypotheses have been proposed and studies are being conducted to understand the pathophysiology of ALS. With a better understanding of the mechanisms that cause the disease, researchers hope to unveil new pathways and molecular targets for novel therapies. In addition to the benefit of expanded and more efficacious treatment options, healthcare professionals believe that better tools and physician awareness for early diagnosis of ALS will help improve the prognosis of ALS patients.
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2 Introduction

2.1 Catalyst

This report is being produced to provide a thorough analysis of the current and the future landscape of amyotrophic lateral sclerosis (ALS) therapeutics in the major markets of the US, France, Germany, Italy, Spain, the UK, Germany and Japan.

Rilutek (riluzole) is the only approved disease-modifying treatment for ALS. There has not been any drug approved for ALS in the past decade, although the market has other off-label drugs which are used to treat the symptoms associated with ALS. Therefore, a high unmet need remains in the market for drugs with a novel mechanism of action which can alter disease progression.

The ALS therapeutic market is anticipated to show a decline in the forecast period, dipping to $70m by 2019. Although the market has several potential product launches, the patent expiry and genericization of Rilutek will restrict market growth.
11.5 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.

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