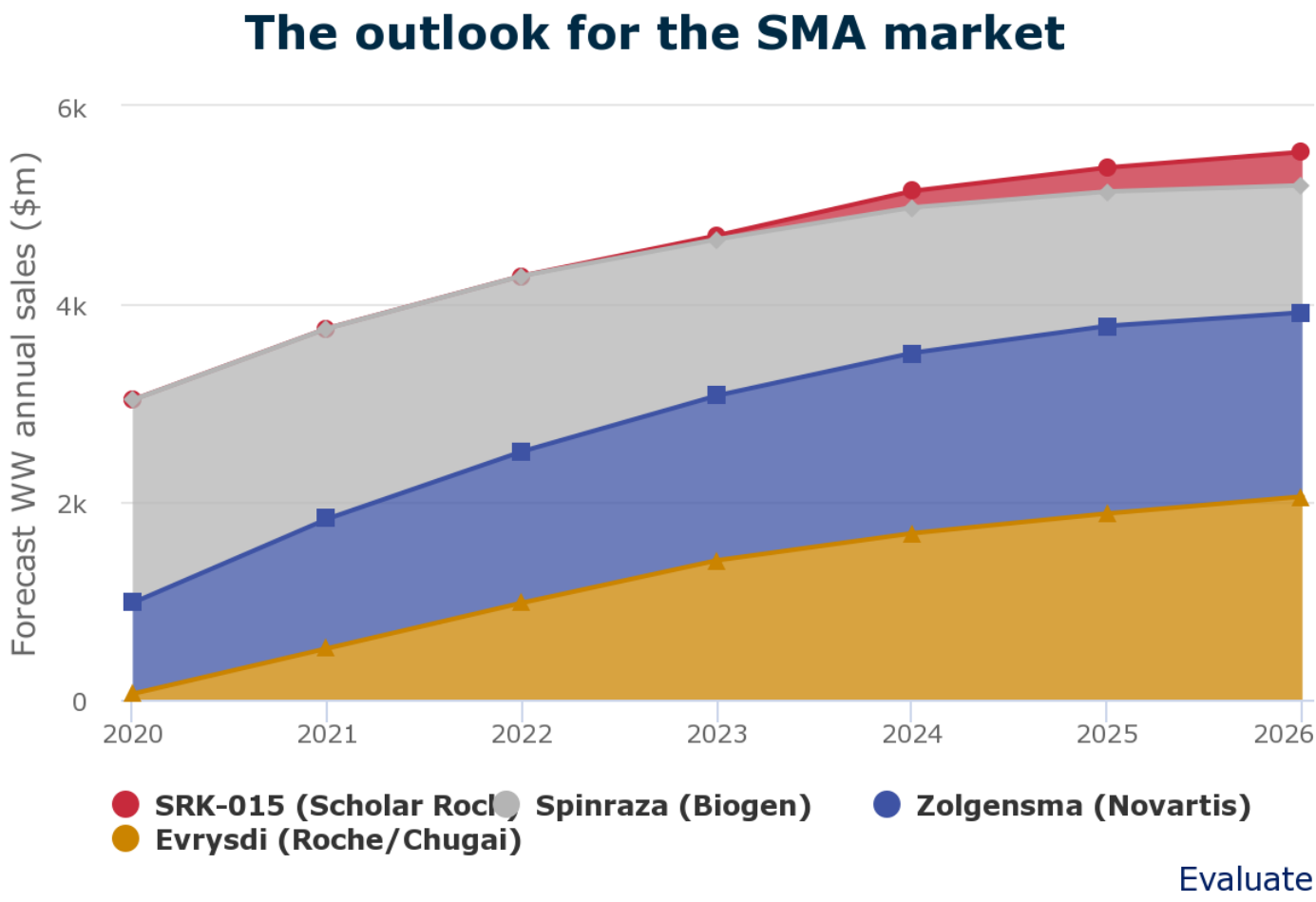
Evaluate Vantage

August 03, 2021

Novartis tries again with intrathecal Zolgensma

Elizabeth Cairns

It has taken nearly two years, but the partial clinical hold placed on the intrathecal formulation of Novartis's Zolgensma has finally been lifted. An intravenous form of the spinal muscular atrophy gene therapy is approved; the intrathecal version is intended to allow treatment of older patients. Novartis is to start a new pivotal trial, Steer, in treatment-naïve patients aged two to 18 with SMA type 2 who are able to sit, but have never walked. More than 100 patients will be randomised to either intrathecal Zolgensma or sham, and efficacy will be evaluated using the Hammersmith Functional Motor Scale-Expanded after a year. Reaching this older population is important: Novartis believes the intrathecal formulation could more than double the number of patients who could be treated with the gene therapy. So far over 1,400 patients have received intravenous Zolgensma worldwide. According to a survey of doctors conducted by SVB Leerink, were the intrathecal Zolgensma to gain approval for older Type 2 patients, around 25% of patients currently on maintenance therapy with Biogen's SMA therapy Spinraza could switch to Novartis's product. Still, intrathecal Zolgensma is not expected to reach market until 2024, even if Steer is a resounding success.



More from Evaluate Vantage

Evaluate HQ
44-(0)20-7377-0800

Evaluate Americas
+1-617-573-9450

Evaluate APAC
+81-(0)80-1164-4754