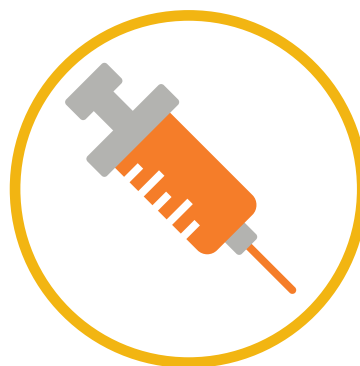


# PIPELINE INSIGHTS REPORT: 3 DRUGS TO WATCH



## RISDIPLAM

If approved, risdiplam would be the first oral therapy to treat spinal muscular atrophy (SMA). There are currently only two FDA-approved drugs for SMA: Spinraza<sup>®</sup> (nusinersen) requires repeated, invasive injections and Zolgensma<sup>®</sup> (onasemnogene abeparvovec), which is a one-time IV-infused gene therapy. In contrast, risdiplam is administered orally once daily.



## VILTOLARSEN

Viltolarsen is in development to treat specific forms of Duchenne muscular dystrophy (DMD). DMD is a rare genetic disorder that affects young boys with the onset of symptoms occurring between three and five years of age. Progressive muscle weakness leads to confinement to a wheelchair by the early teen age years. If approved, viltolarsen would be the third exon-skipping drug for DMD, and the second specifically for the 8% of patients with a specific mutation.



## TRODELVY<sup>™</sup> (SACITUZUMAB GOVITECAN-HZIY)

Trodelvy was FDA approved on April 22, 2020 to treat adult patients with metastatic triple-negative breast cancer (TNBC) who have received at least two prior therapies for metastatic disease. TNBC is an aggressive breast cancer that accounts for up to 20% of all breast cancer cases.

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