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FDA approves Itvisma, the first gene-replacement therapy for SMA patients aged 2 or older

Market Event Summary

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FDA approves Itvisma (intrathecal onasemnogene abeparvovec)

Background

- Novartis / Novartis Gene Therapies' Zolgensma (intravenous onasemnogene abeparvovec) was approved for SMA patients under the age of 2 in 2019 in the United States and in 2020 in Europe and Japan.
- However, **a significant unmet need persisted for SMA patients aged 2 or older**. Treatment options for this population were limited to chronic therapies, including Biogen's intrathecal nusinersen (which carries a considerable administration burden) or Genentech's oral risdiplam (which poses compliance challenges). In older, heavier SMA patients, the weight-based intravenous dose cannot be safely administered because of the toxicity risks.
- To address this gap, Novartis developed Itvisma, the same molecule as Zolgensma but **a different dose delivered intrathecally directly into the spinal cord**, targeting the motor neurons more efficiently at a lower dose.

Event

- On November 24, 2025, **the FDA approved intrathecal onasemnogene abeparvovec (brand name Itvisma)** for all SMA patients aged 2 or older with a confirmed mutation in the *SMN1* gene, marking a significant expansion as the first and only gene-replacement therapy for this broader population.
- The FDA approved Itvisma based on the results from the **Phase 3 STEER trial and the Phase 3b STRENGTH** trial.
  - Treatment-naive patients in the STEER trial demonstrated a 2.39-point improvement on the Hammersmith Functional Motor Scale – Expanded (HFMSE), compared with 0.51 points in the sham group.
  - Patients switching to Itvisma from nusinersen or risdiplam in the STRENGTH trial showed stabilization in motor function over 52 weeks, with 90% of patients with a median increase of 4 points on the HFMSE scale.

Clarivate's takeaways

Commercial outlook

Itvisma's approval will expand the SMA gene therapy market, with all SMA patients now being eligible for Novartis's treatment. We forecast **peak-year sales of onasemnogene abeparvovec (Zolgensma and Itvisma) to exceed $1 billion**.

Market access and reimbursement

With newborn screening implemented across all 50 states in the U.S. and **increasing payer acceptance** of high-value rare-disease therapies, Itvisma has the potential to reach a large percentage of the SMA patient population.

Uptake constraints

Given its intrathecal administration, Novartis might need to set up **specialized centers to administer** Itvisma, a step that could take time and delay rapid uptake. Itvisma will also **compete with nusinersen and risdiplam** for the same subset of SMA patients aged 2 or older.

About the author

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Dr. Priyadarshini has 9 years of experience in market assessment, competitive intelligence, primary market research, market forecasting, analogue and indication assessment, disease portfolio optimization. Dr. Priyadarshini trained as a molecular biologist; her research focused on the interplay between tumor suppressor proteins and oncoproteins. She received her doctorate from the National Institute of Immunology in New Delhi, India.

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