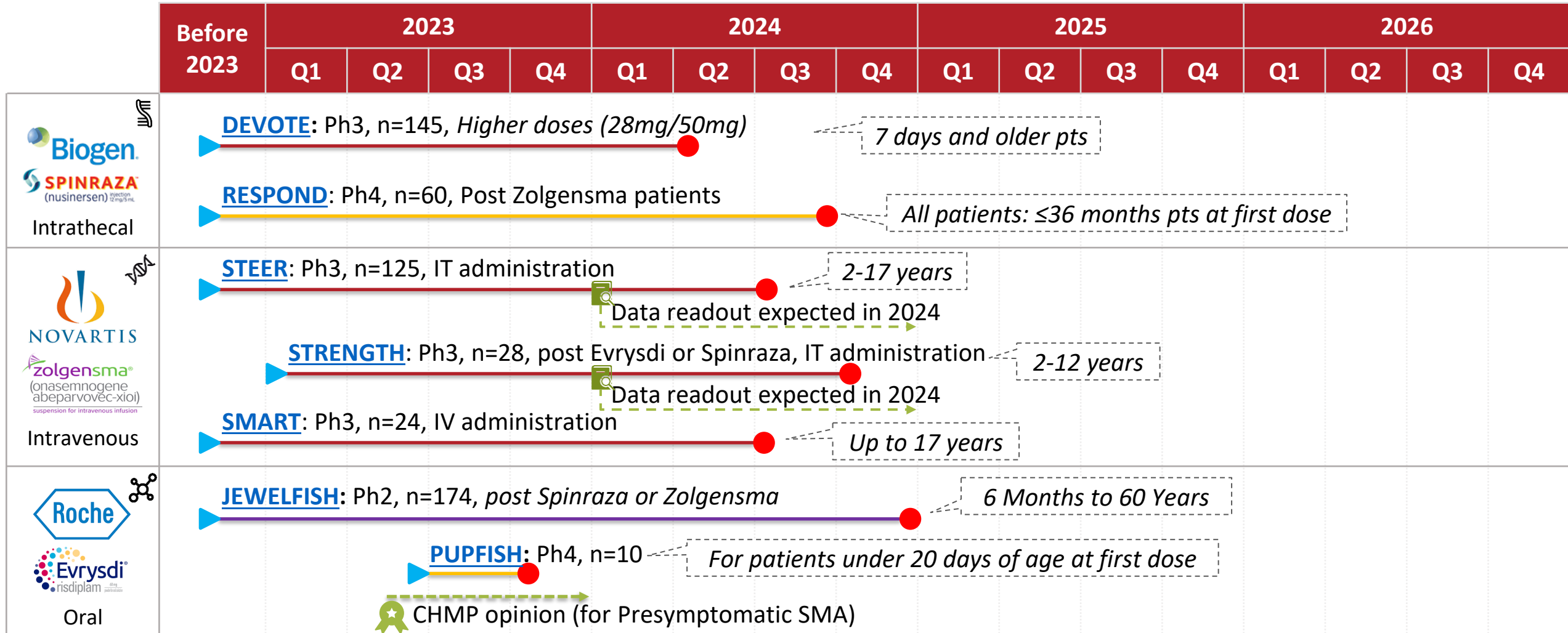




Novel and Emerging Treatment Options in Spinal Muscular Atrophy



Marketed products are being evaluated for age expansion, dose increase, and in previously treated SMA patients



Source: Atacana analysis, CT.gov, Citeline, Company PRs
 Note: Key events for US & EU till 2026 have been included


 Antisense
 Oligonucleotides


 Gene
 Therapy


 Small
 Molecule

 Phase 4
 Phase 2

 Phase 3
 Phase 2/3

 Study Start
 Study PCD
 Time Range

 Data Readout
 Regulatory

Trends

Spinraza

- Evaluating higher doses for less frequent dosing
- Potentially for patients who had inadequate response to Zolgensma

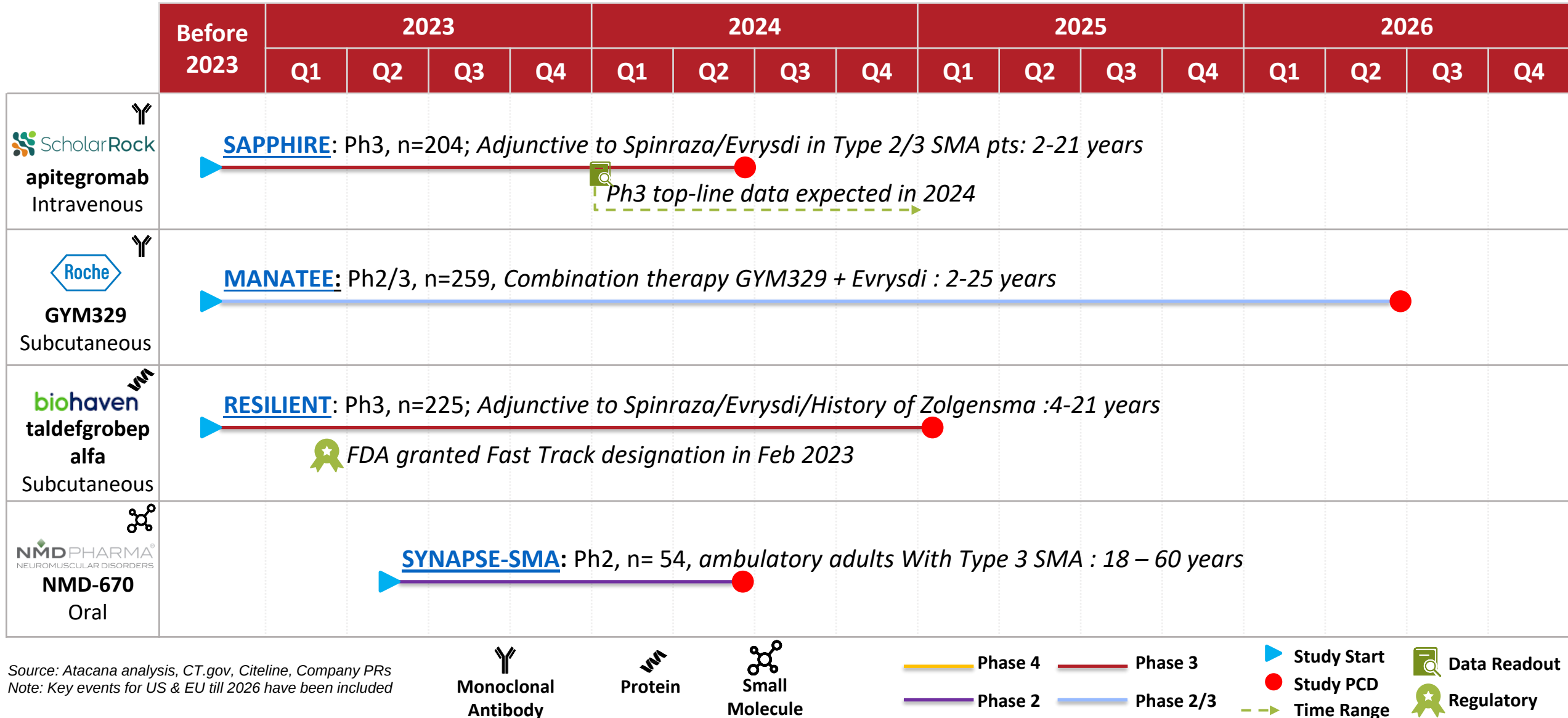
Zolgensma

- Pursuing label expansion in older pediatric patients (2-17y) with intrathecal formulation

Evrysdi

- Exploring as an alternative option for patients after Spinraza / Zolgensma
- Potential first oral treatment options for pre-symptomatic babies with SMA in the EU

Muscle enhancing pipeline drugs are evaluating combination approach with SMN up-regulators to further improve patient outcomes



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 Note: Key events for US & EU till 2026 have been included

Trends

Myostatin inhibitors

- Scholar Rock's apitegromab is expected to be the first muscle-directed add-on treatment to enter the SMA market
- Apitegromab, GYM329, and taldefgrobep alpha target myostatin to enhance muscle growth and muscle function

CIC Inhibitor

- NMD-670 is a skeletal muscle specific chloride ion channel 1 (CIC-1) inhibitor; it is the only clinical CIC-1 targeting product
- NMD PHARMA expects CIC inhibition will lead to restoring neuromuscular function



Directional Clarity
Actionable Insights
Exponential Advantage

If you need help developing a
winning strategy, email us at info@atacana.com