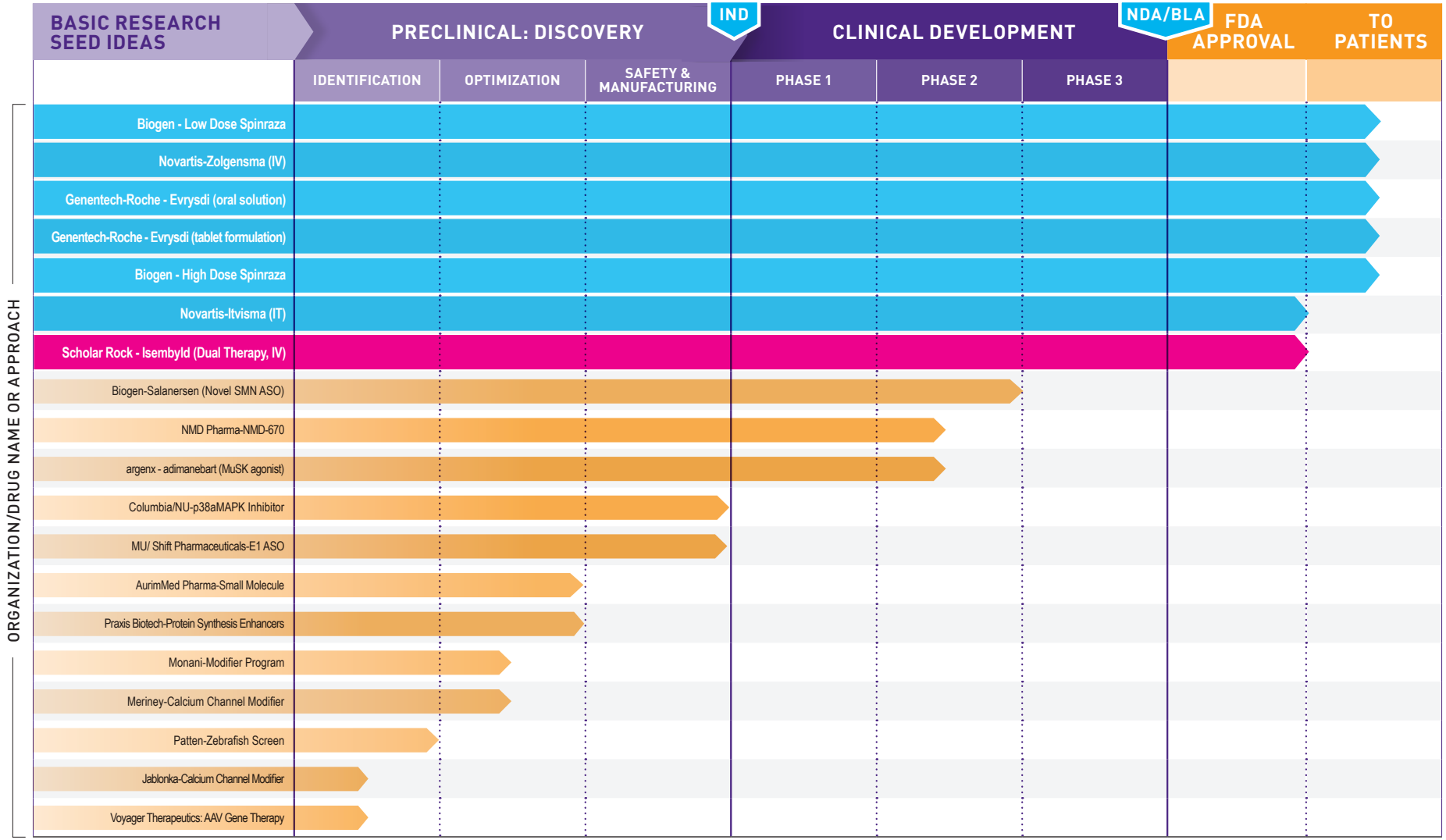
SMA DRUG PIPELINE

We're funding and directing research with more breadth and depth than ever before. We know what we need to do to develop and deliver new therapies, which could also work in combination, to reach our goal of treatments for all ages and types. And we're on the verge of further breakthroughs that will continue to change the course of SMA, and eventually lead to a cure.



BLA = Biologics License Application

IND = Investigational New Drug

NDA = New Drug Application

SMN Enhancing

Muscle Directed

Investigational (Preclinical)

Last updated: September 2026