

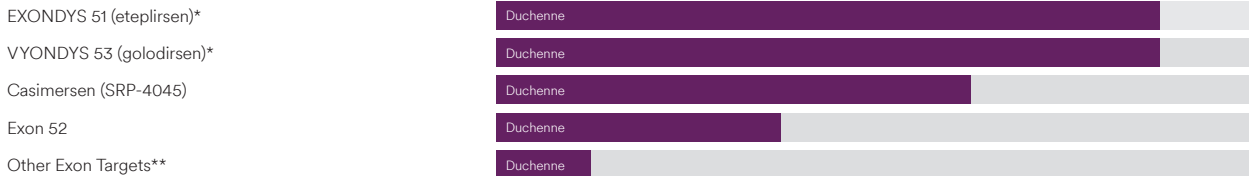
DISCOVERY

PRE-CLINICAL

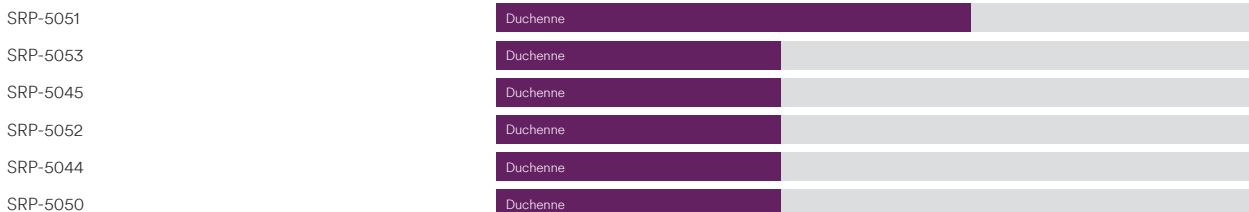
CLINICAL

COMMERCIAL

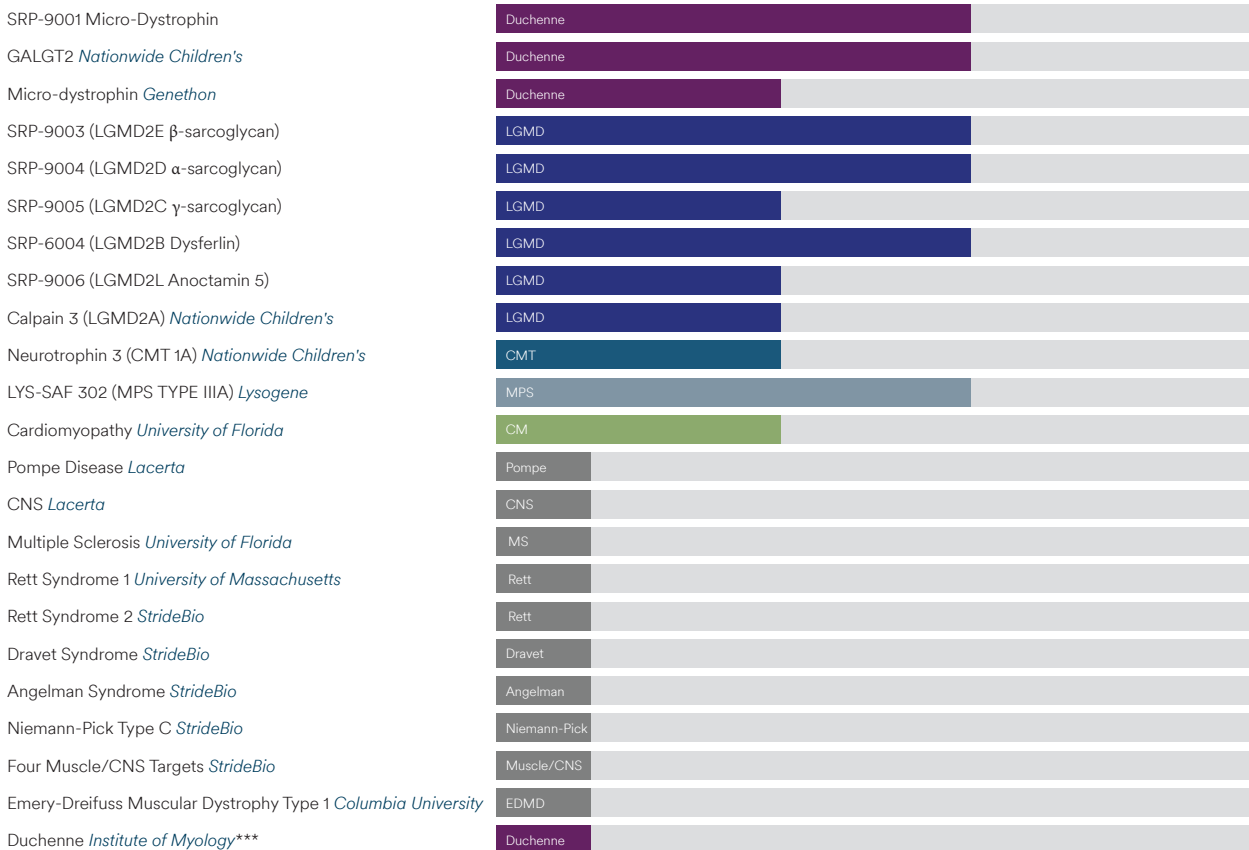
## RNA-TARGETED THERAPIES PMO



## RNA-TARGETED THERAPIES PPMO



## GENE THERAPY



## GENE EDITING



\*Candidate received accelerated approval in the U.S., confirmatory studies required

\*\*Other exon targets in development: 43, 44, 50, and 55

\*\*\*Gene therapy and PPMO

Duchenne - Duchenne muscular dystrophy  
 EDMD - Emery Dreifuss Muscular Dystrophy  
 LGMD - Limb-girdle muscular dystrophy  
 MPS - Mucopolysaccharidosis IIIA  
 CMT - Charcot-Marie-Tooth disease  
 CNS - Central nervous system  
 CM - Cardiomyopathy  
 MS - Multiple Sclerosis