



Our Pipeline

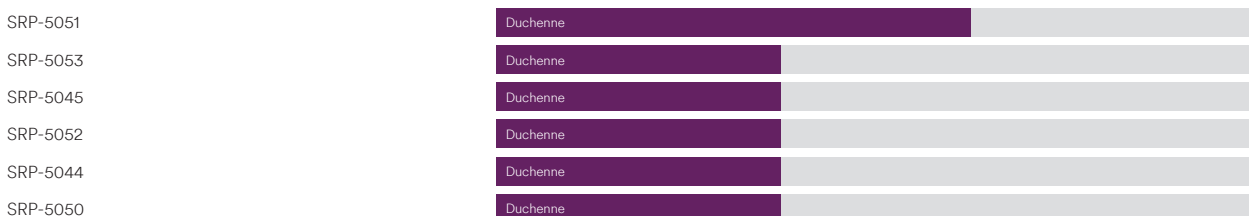
Information is current as of 1/13/2022, updates are made on a quarterly basis



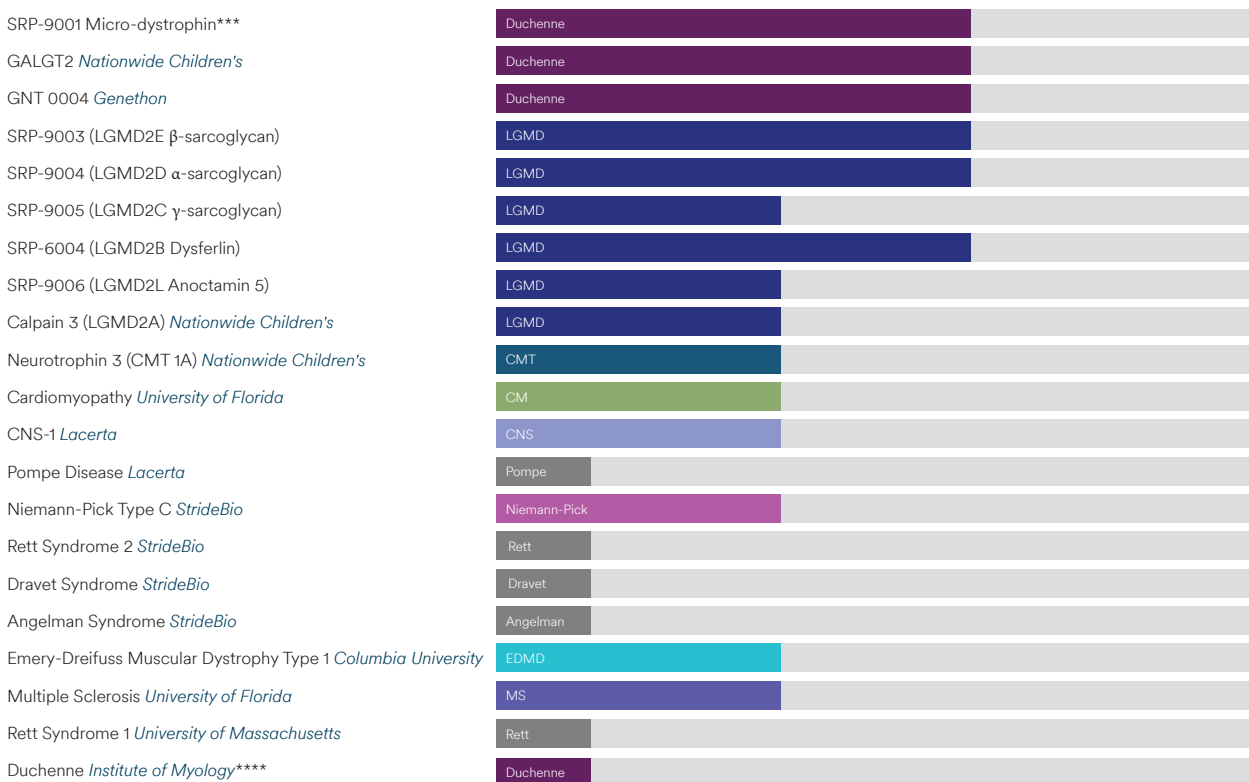
RNA-TARGETED THERAPIES PMO



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

***Roche has the exclusive right to launch and commercialize SRP-9001 outside the United States

****Gene therapy and PPMO

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