



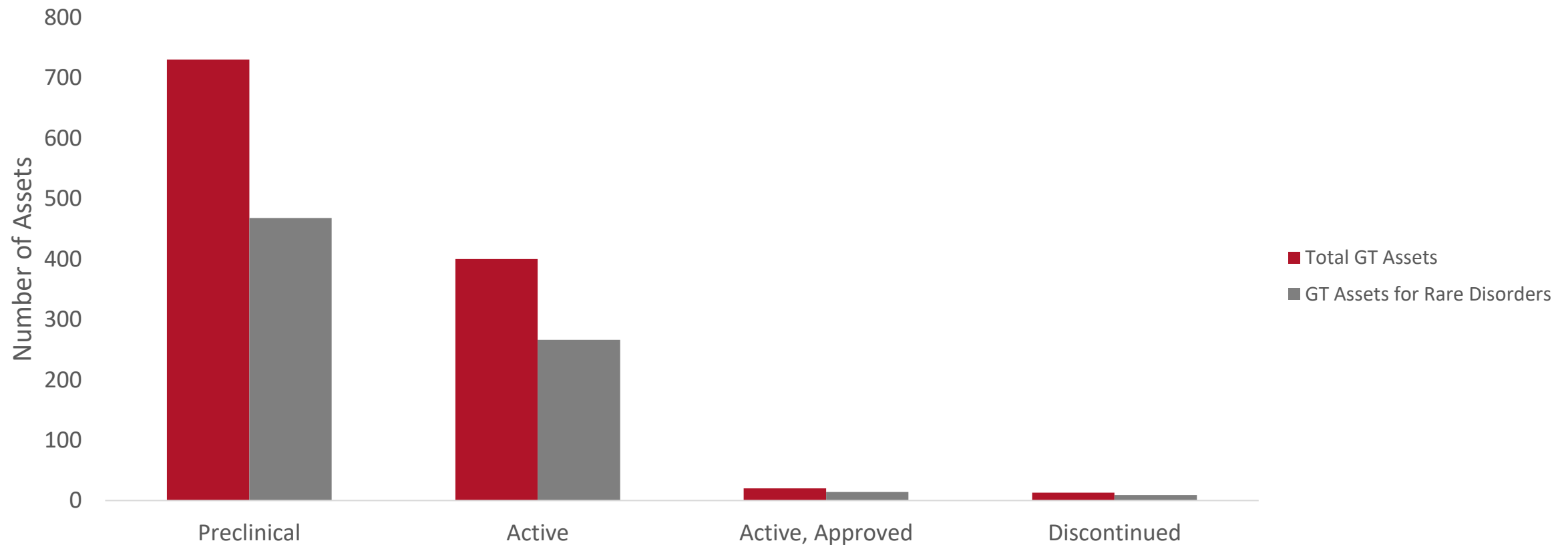
GENE THERAPY
Beacon
Targeted Therapies

Insights for GT Rare Disorders 2021: Drug Landscape of GT for Rare Disorders 2020

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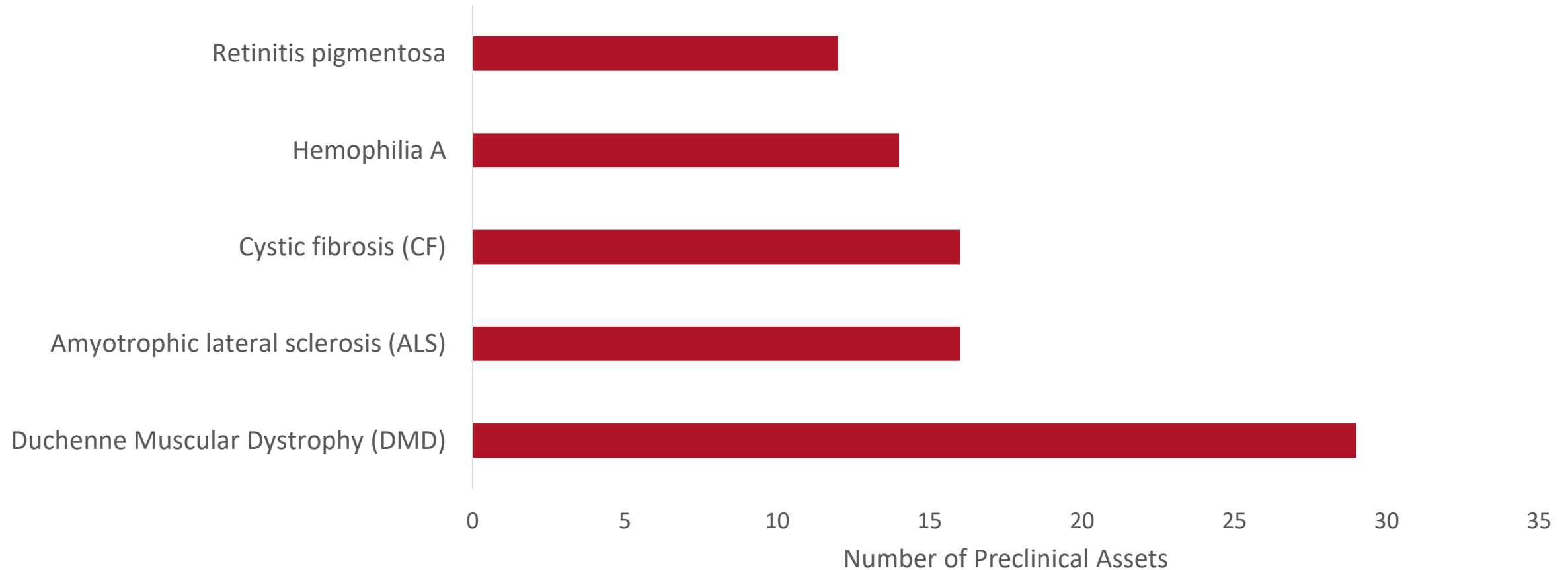
Therapeutic Landscape for GT RD

Drug Status Distribution of Gene Therapies for Rare Disorders Vs Overall Gene Therapy for Non-Oncology Landscape



Therapeutic Landscape for GT RD

Top Five Disclosed Rare Disease Indications in the Preclinical Landscape



Summary

- Of the total 1,163 assets on Beacon for genetically driven non-oncology disease indications, 65% of these therapies are in development for a rare disorder
- Of those assets that have been approved, 70% have been for a rare disorder
- The landscape is still dominated by rare diseases, with the top 5 disease indications for preclinical assets all being rare disorders (Duchenne muscular dystrophy, ALS, cystic fibrosis, Hemophilia A and retinitis pigmentosa)
- The indications named above comprise 33% of the preclinical landscape