



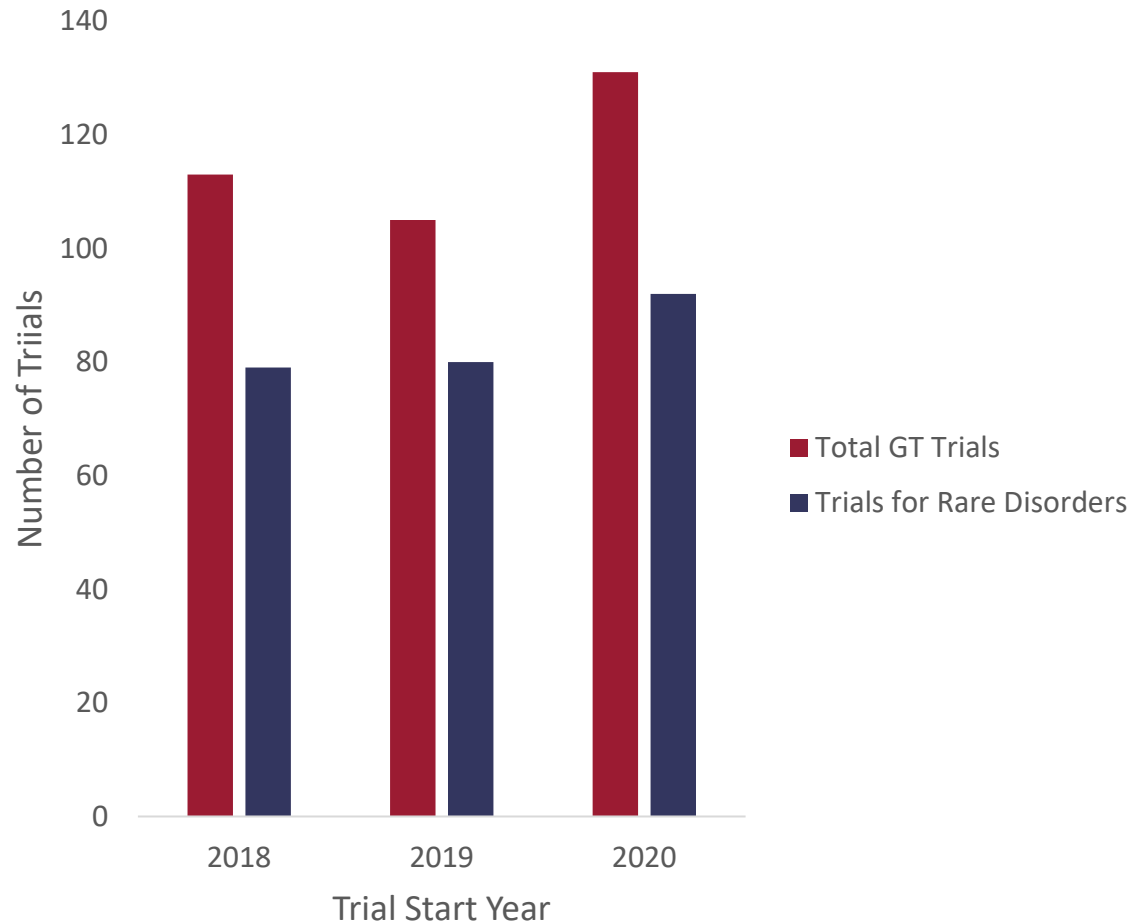
GENE THERAPY
Beacon
Targeted Therapies

Insights for GT Rare Disorders 2021: Trial Landscape for GT Rare Disorders 2020

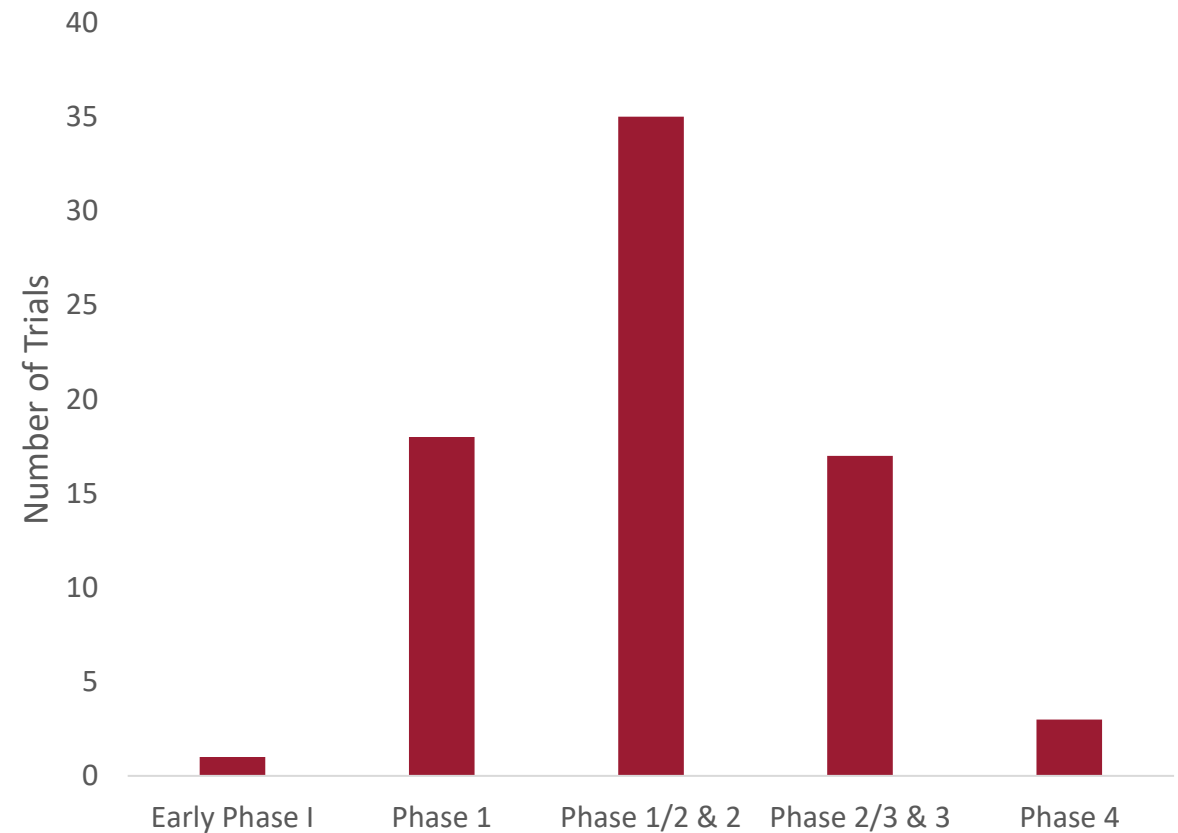
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2020 Trial Landscape

Trials Initiated in the Last Three Years

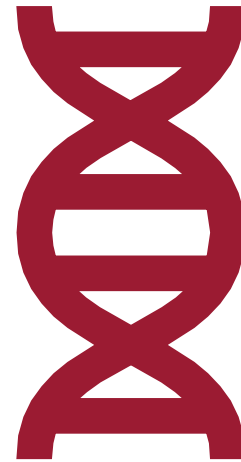
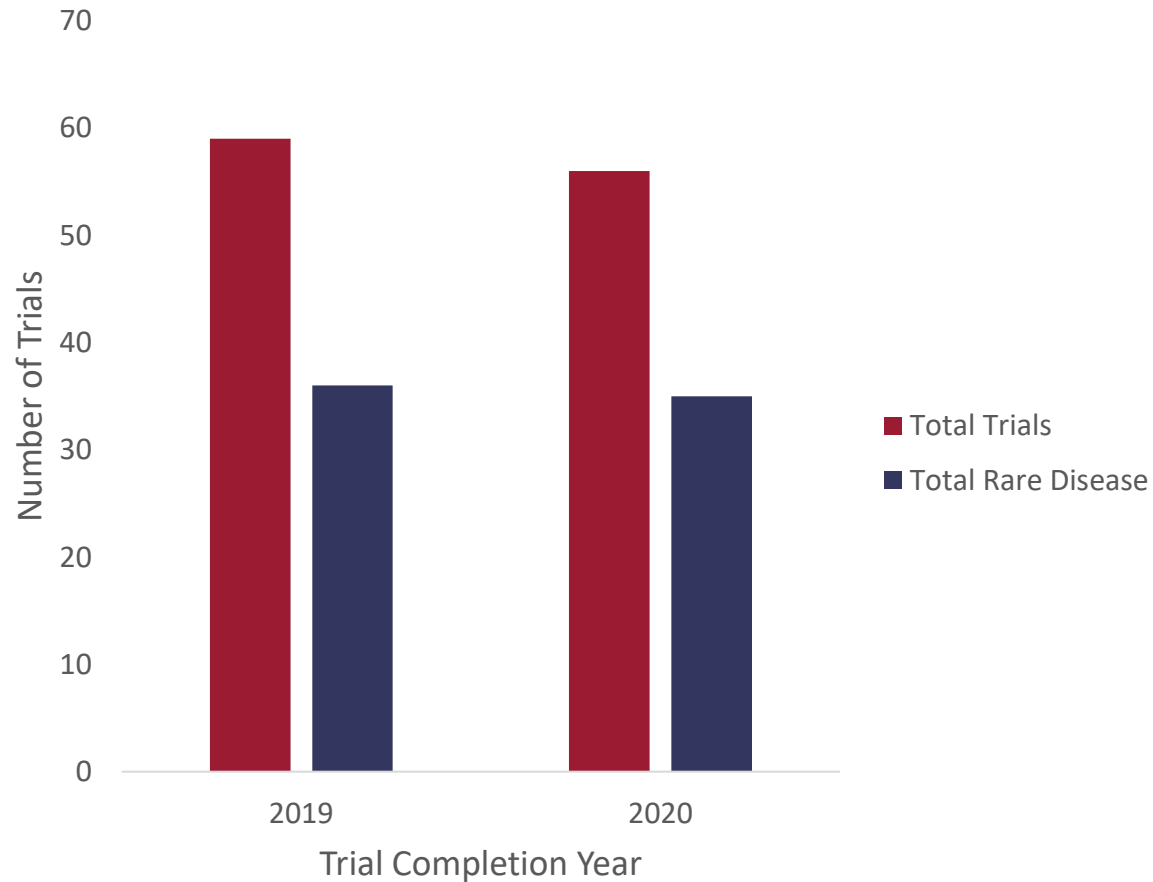


Phase Distribution of GT Trials for Rare Disorders Initiated in 2020



2020 Trial Landscape

Trials Completed in 2019 vs 2020



**30 New Gene
Therapy Assets
Entered the Clinic in
2020 for a Rare
Disease**

2020 Trial Landscape

- Even with COVID-19 effecting many studies, gene therapy for rare disorders saw a 15% increase in trials initiated in 2020 compared to 2019
- Majority of trials initiated (47%) were in phase 1/2 & 2
- There was a decrease in gene therapy trials completed overall for 2020, with a decrease of 5% compared to 2019. Specifically, gene therapy trials for rare disorders trials completed decrease by 3%