

planned 2022 milestones – focus on regulatory milestones and commercialization

Beta-thalassemia

Ad Comm
DATE TO BE ANNOUNCED

FDA PDUFA date
AUGUST 19, 2022

Commercial launch
2022

Cerebral Adrenoleukodystrophy

Ad Comm
DATE TO BE ANNOUNCED

FDA PDUFA date
SEPTEMBER 16, 2022

Commercial launch
2022

Sickle Cell Disease

*Complete manufacturing
of commercial drug
product validation lots*
MID-2022

*Confirm vector and drug
product analytical
comparability*
Q4 2022

BLA submission
Q1 2023

*Currently evaluating what impact, if
any, partial clinical hold may have on
BLA target submission date*