IQVIA Cell And Gene Therapy (CAGT) Center:
Core Services for CIRM Awardees

I. Technology Transfer, Process Scale-up, Manufacturing & CMC Support

- ***Description:*** The CAGT Center supports the development of manufacturing process and controls strategies for early and late stage studies. The CAGT Center partners with City of Hope to offer specialized scale up and early phase manufacturing services and with WuXi AppTec for late phase manufacturing customized to cell and gene therapies. Services include cell expansion / cell harvest GMP compatible process development, GMP reagent sourcing, GMP compatible scale up, protocol development, assay development, and SOP QC/QA.

- ***Benefits:*** CMC is often a key rate-limiting factor for initiating a clinical study, so early evaluation of CMC needs and development of a comprehensive and actionable plan serves to minimize time, risk and cost of therapeutic development.

II. Preclinical / Nonclinical Research

- ***Description:*** The CAGT Center can help plan for generating the necessary preclinical evidence to support the IND approval process. The CAGT Center partners with Charles Rivers Laboratories to provide capabilities in discovery, animal models, toxicology, and efficacy studies customized to the novel properties of each cell and gene therapy products.

- ***Benefits:*** Conducting the necessary early research studies will ensure that essential preclinical evidence is collected to minimize gaps in the IND application and decrease the risk of future delays and costs.

III. Asset Development Strategy

- ***Description:*** The CAGT Center supports innovative development strategies by anticipating clinical requirements at the preclinical stage. Integrated asset development planning occurs throughout the development timeline and includes services such as indication selection, biomarker strategy, and nonclinical/clinical planning prior to IND submission and clinical trial modeling after IND approval.

- ***Benefits:*** For manufacturers preparing for IND, there is often a mismatch between existing data and FDA requirements. A development strategy will identify how to address mismatches and plan for successful INTERACT, pre-IND, and IND meetings. Beyond IND approval, an integrated asset development plan will incorporate clinical, commercial, and regulatory insights to improve the probability of the asset's commercial success.

IV. Regulatory Strategy and Operational Services

- ***Description:*** The CAGT Center can support a wide range of activities across the evolving cell and gene therapy regulatory landscape from regulatory strategy, meetings (INTERACT, pre-IND, etc.) with regulatory authorities, and preparing and submitting INDs, BLAs and other regulatory

submissions. In addition, the CAGT Center can help your technology secure special designations (orphan, breakthrough, RMAT, accelerated approval, pediatric rare disease).

- ***Benefits:*** A comprehensive regulatory strategy will enable a technology to achieve key regulatory milestones in an efficient and timely manner and streamlines approval in target geographic regions. A special designation can further expedite the road to approval, decrease evidence needed, increase interactions with the FDA, and increase asset value.

V. Target Product Profile (TPP) Development

- ***Description:*** A TPP is a summary of the aspirational product label and performance requirements. The CAGT Center supports the development of a TPP by understanding the disease target and associated commercial, clinical and regulatory needs. Through the TPP development, the CAGT Center will highlight points of scientific differentiation and assist in translating these differentiating features into measurable Product Profile attributes.

- ***Benefits****:* The TPP forms the basis for a clinical development program that delivers on the required commercial goals for the product and that is acceptable to regulatory agencies. Additionally, a clear TPP aids in communication of the product strategy to various stakeholders (e.g., investors, regulatory bodies, strategic partners, etc.). Finally, the TPP will be used to guide forecasting, and to facilitate obtaining early input from physicians and payers on the commercial product.

VI. Pharmacoeconomics Services

- ***Description:*** The CAGT Center supports the development of objective evidence of economic value to help technologies optimize market access outcomes. These services include developing and testing value drivers and overall value proposition for key market access stakeholders, developing an evidence strategy to optimize evidence for that value proposition, and leveraging clinical data, secondary, and primary health-economic research to develop the overall evidence package.

- ***Benefits:*** Early commercial and value proposition planning will enable CIRM grantees to navigate market access challenges, particularly those arising from payer and provider concerns around cost and evidence. Establishing a compelling value proposition for key market access stakeholders will help CIRM grantees achieve optimal pricing, market access, and uptake.

VII. Clinical Operations

- ***Description:*** The CAGT Center offers end-to-end clinical trial services, including design and execution, to bring cell and gene therapies to patients faster. Support ranges across site, investigator, recruitment, regulatory, testing and safety activities. The CAGT Center leverages the experience and capabilities of IQVIA, one of the world's leading CRO's, to ensure efficient and quality clinical trial execution.

- ***Benefits:*** There are many complexities that exist within cell and gene therapy clinical development, yet the execution of a well-designed clinical trial will enable a product to

accelerate timelines to market, optimize costs, mitigate risks, and increase probability of technical success.