[395] IN VIVO GENOME-EDITING FOR RARE PEDIATRIC DISEASES

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ABSTRACT TEMPLATE for Company Presentations

Questions for Biotech/Pharma; Medical Devices and Health IT/Digital Health categories are:

- **Investment Rational**
  LogicBio Therapeutics, Inc. (LOGC) is a gene therapy company, based in Cambridge, MA, with a mission to develop lifelong cures for serious, early-onset rare diseases with great unmet medical need. We are initially focusing on pediatric patients suffering from diseases for which we believe our proprietary technology platform, GeneRide, can provide significant advantages over current therapeutic approaches.

- **Business Strategy**
  The company is differentiated from other gene therapy approaches by a platform technology (GeneRide™) developed in the laboratory of Professor Mark A. Kay at Stanford University. Our mission is to transform the lives of patients with devastating genetic diseases by building the leading integrated genetic medicine company focused on developing and commercializing therapeutics based on our GeneRide platform.
- **Core Technology**
  GeneRide is our genome-editing platform that harnesses homologous recombination to provide precise, site-specific, and durable integration of a corrective gene into the chromosome of a host cell. This provides a more durable approach than gene therapies that do not integrate and lose their effect as cells divide. These benefits make GeneRide well-positioned to treat genetic diseases, particularly in pediatric patients.

- **Product Profile/Pipeline**
  We are developing LB-001 to treat methylmalonic acidemia (MMA), a life-threatening rare disease characterized by the toxic buildup of metabolites starting in early childhood. We continue to develop additional programs focusing on other rare diseases that can be addressed with genomic editing of hepatic cells. Initial PoCs have been published in diseases such as hemophilia B and Crigler-Najjar syndrome.

- **What's Next?**
  Key elements of our strategy include: 1. advance LB-001 through clinical trials and ultimately commercialization; 2. pursue additional indications addressed by targeting the liver; 3. collaborate to realize the full potential of GeneRide; 4. build an exceptional team and organization; and 5. maintain our scientific leadership.