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PRESENTATION TITLE: Mendelian genes as rare and common disease targets.

ABSTRACT: Precision medicine represents an effort to identify the mechanistic causes of disease in individual patients and target treatments to those causes. Dramatic advances in resolving the genetic causes of disease have created new opportunities for precision medicine, though profound challenges remain. Key amongst those challenges are the diversity of underlying causes of disease and limitations of available pre-clinical models. Here I outline approaches for developing precision medicines for rare genetic diseases and opportunities to generalize those treatments to subsets of more common diseases.

BIOGRAPHY: David Goldstein, PhD: Human geneticist renowned for research on human genetic diversity, the genetics of disease, and pharmacogenetics. Recently, was Director of the Institute for Genomic Medicine, and Professor of Genetics and Development at Columbia University, with the mission to integrate genetics and genomics into research, patient care, and education. Previously directed Duke University's Center for Human Genome Variation. Discovered several disease-causing genes and syndromes, specifically neurological and infectious diseases. Additionally, served as advisor to numerous pharmaceutical companies, including as AstraZeneca's chief genomics adviser and led an integrated initiative focused on the discovery of new targets and biomarkers linked to molecular mechanisms of disease across multiple therapy areas. Co-Founder of Praxis Precision Medicines, a public clinical-stage biopharmaceutical company translating genetic insights into the development of therapies for patients affected by central nervous system ("CNS") disorders characterized by neuronal imbalance. Currently, CEO of Actio Biosciences