Neuromuscular Banking, Drug Development, Gene Therapy, and Commercialization

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Prof. Eric Hoffman, Professor and Associate Dean, School of Pharmacy and Pharmaceutical Sciences, Binghamton University – State University of New York, the CEO ReveraGen BioPharma, and the CEO AGADA Biosciences was the last speaker of BAS Plenary Session. Prof. Eric Hoffman presented on "Neuromuscular banking, drug development, gene therapy, and commercialization". Neuromuscular banking is the long-term storage of biological materials from neuromuscular patients for research or molecular diagnostic purposes. The biological material could be DNA (blood) or pathological tissues. Knowledge of the underlying causes of many neuromuscular disorders has been defined by genetics approaches, including most forms of muscle and nerve disease. The key patient material to define the primary cause of the genetic disease is peripheral blood DNA, and the banking and analyses of patient DNA are now readily analyzed by gene sequencing approaches to define the primary gene and protein defect. Understanding of disease pathophysiology (downstream biochemical and cellular consequences of the primary gene defect) is often more complex, and requires molecular studies of proteins, mRNA, microRNA and cell dysfunction. Archived pathological tissues are critical for such studies, ideally tissues kept in deep frozen storage. An example of utilization of patient archived materials for definition of molecular pathophysiology of nuclear envelope disorders is provided, enabling insight into the varied clinical phenotypes associated with different mutations. Translation of knowledge of a disease's genetics and pathophysiology into effective therapies can take many paths. Prof. Hoffman described a successful precision mutation-targeted therapy in muscular dystrophy and the use of molecular approaches to develop a dissociative steroidal anti-inflammatory. He mentioned that these two drugs were developed under academic/private partnerships but utilized two different development and business models that were both highly cost-effective and robust. One drug was licensed from an academic group (National Center of Neuroscience Psychiatry, Japan) by an established pharmaceutical company (Nippon Shinyaku Pharma), with parallel trials in Japan and USA led by academic clinical trial networks. This led to an 'accelerated approval pathway in both the US and Japan. The other drug was developed by ReveraGen BioPharma, an academic spin-off that partnered with multiple government agencies and non-profit foundations to carry out scientifically robust clinical trials in the same academic clinical trial networks. These three cases illustrate the importance of biobanking, establishing academic clinical trial networks, and innovative business models in developing new therapies for genetic disorders.