Hemophilia CA*NEXT*ions

A Novel Therapies Access Summit Presented by the Hemophilia Council of California
Executive Summary of November 2020 Summit

This event brought decision makers together for a dynamic, interactive conference with discussions on health policy coverage and payment, as it relates to gene therapy for hemophilia. It informed key stakeholders – including policy makers, public and private payers, health systems, healthcare providers and advocates from the patient community – about the potential impact of gene therapy on California's health care system. The goal of this event was to educate stakeholders, spark dialogue about implications for California and identify potential gaps in access to gene therapy.

Session #1: Gene Therapy Overview
Speaker: Dr. Leonard A. Valentino, CEO of the National Hemophilia Foundation
Session Content: Overview of gene therapy, including how hemophilia treatment has improved in efficacy and ability to extend patients' lifespans and quality of life. Overview started in the 1950s, when FFP cryoprecipitate injections were first introduced to supplement the low factor levels in patients' blood and treat bleeds. Overview ended with present day, when gene therapy trials have shown an amazing ability to keep factor levels stable and prevent bleeding without regular injections. Dr. Valentino also discussed restrictions and limits to gene therapy accessibility. Limits included age minimums, liver health, and immune response against the viral vector used to transfer the genetic material. Some realistic possible outcomes of new therapies included:

- Greatly reduced use of blood factor product- an expensive, lifesaving medication-
- Reduced joint damage, and resulting chronic pain, from bleeds
- Improved mental health
- Fewer emergency room visits to treat dangerous bleeds
- And a possible route to a cure for hemophilia (pending more information on duration of gene therapy efficacy)

Dr. Valentino also discussed the importance of the Hemophilia Treatment Center network in distribution of gene therapy and long-term monitoring of patients, which will be vital in implementing gene therapy. Possible risk factors and possible complications were also discussed.

Session #2: Patient Perspective Panel
Session Content: Panelists discussed their personal hemophilia story. Panelists included: **Maxwell Feinstein**, who had 15 years' experience on a prophylaxis regimen; **Nicolas Kell Macken**, who received gene therapy on a clinical trial; and **Rebecca Buchmiller**, the mother of a 17-year-old with severe hemophilia. Panel moderated by **Randall Curtis**, MBA, a long-time advocate in the bleeding disorders community with extensive experience in collecting data for studies such as utilization reports and patient reported outcomes. Maxwell noted how joint damage from his hemophilia challenged him. Both Maxwell and Nicolas described difficulties staying adherent to their treatment regimens, which often required more than once a day injections, especially during their teen years. Rebecca described the incredible advancements in treatment efficacy and ease of administration over the time between her son's first diagnosis as a small child up until now. All three panelists highlighted the mental health challenges of dealing with a disorder that requires frequent injections and limits on physical activity yet can still result in spontaneous bleeding and joint damage. The panel wrapped up with Nicolas describing how gene therapy changed his life. He went from needing injections multiple times a week to zero injections, and this has opened up new possibilities he had not imagined possible while he was on prophylaxis, a typical factor treatment.

Session #3: Access to Gene Therapy through Novel Financing Models
Speaker: Dr. James Kenney, Founder and President of JTKENNEY, LLC, a managed care pharmacy consulting practice
Session Content: Overview of possible financing models to pay for gene therapy. Dr. Kenney discussed challenges for payers and payer concerns, such as a lack of head-to-head comparisons since gene therapy is so new. Questions about durability, post treatment monitoring and patient turnover were also discussed, among others. He discussed key value drivers for gene therapy, including medical cost offsets, such as fewer ER visits, and savings from a drop in factor usage due to durability of treatment. Then Dr. Kenney reviewed different possible types of insurer payment methods for gene therapy products and various contract design options. Some of the payment methods included value-based contracts and outcome-based contracts, which determine payment based on the efficacy of the treatment/duration of treatment efficacy. Dr. Kenney also discussed some of the current gene therapies available for other conditions and how they are covered. Which patients might qualify for gene therapy was also covered. Dr. Kenney finished the session with a discussion of savings opportunities with gene therapy and how gene therapy may interact with Medicare and Medicaid.

For more detailed notes and links to video clips from the presentations, please contact Lynne Kinst at (916) 572-7771 or lkinst@hemophiliaca.org. Mark your calendar now and plan to join us for CA*NEXT*ions Summit 2021 on November 9th. More information to come.