

# MEETING AGENDA

## May 17

**12:30 - 1:30 Lunch**

**1:30 - 1:45 Opening and welcome**

*An introduction to the biggest question for the gene therapy/gene editing industry: what's the commercial model for a brand-new industry that can't play by the rules of traditional therapeutics?*

**1:45 - 2:45 Launching one-time therapies**

*Vanishingly few gene therapies have been launched in the US. What lessons can those few teach us? We'll hear the top dos and don'ts to drive commercial success.*

Speakers:

- **Moderator: Pierre Jacquet**, Vice Chairman, Global Healthcare, Managing Director, L.E.K. Consulting
- **Sarah Pitluck**, Founder, SP Consulting and former VP, Global Pricing & Reimbursement Strategy, Spark Therapeutics
- **Mani Foroohar**, MD, Senior Managing Director, Biotechnology analyst, SVB Securities

**2:45 – 3:15 Networking break**

**3:15 - 5:15 Commercializing gene therapies: case studies on big questions**

*We'll split into small groups that each address a priority commercial question facing gene therapies today. Using fictionalized case studies, each group will brainstorm and then develop potential solutions to the challenge question, with help from facilitators from RE, LEK, and Orsini. Findings will be presented back to the group for feedback and iteration.*

- How do you price one-time therapies for more common diseases?
- How can you track the long-term efficacy of a gene therapy?
- What's required to advance a fast-follower gene therapy?

**6:30 Cocktail reception and dinner**

# May 18

## 7:00 – 8:00 Breakfast

**8:00 - 9:00 The View from CMS: After Aduhelm...a sea change in coverage decision making?** *CMS is the wild card for gene therapy – the biggest payer in the US and the most opaque. We'll hear from CMS leaders on how they're thinking about providing access to these new therapies, discuss the impact on innovations, and offer them alternative solutions.*

Speakers:

- **Roger Longman**, Chairman and Co-founder, Real Endpoints
- **Vinod Mitta**, Medical Officer, Center for Medicare and Medicaid Innovation (CMMI)
- **Will Shrank**, Advisor to CMMI and Advisory Partner, Andreesen Horowitz, former chief medical officer, Humana

## 9:00 - 10:00 Value-based agreements – state of the art

*Value-based agreements are one mechanism to drive payer demand for transformative gene therapies, but adoption en masse has remained challenging. In this session, speakers will review what's broadly worked in past risk-sharing agreements, what's different about risk-sharing for gene therapy/gene editing medicines, and how risk-sharing agreements can be structured to solve at least some access challenges.*

Speakers:

- **Jeff Berkowitz**, CEO, Real Endpoints
- **Kristen Wolff**, VP Market Access, bluebird bio
- **Michael Sherman**, CMO, Point32Health
- **Jane Barlow**, Chief Clinical Officer, Real Endpoints and Senior Advisor, Tufts NEWDIGS

## 10:00 – 10:30 Networking break

## 10:30 - 11:45: The Payer's Point-of-View

*The challenges for payers are obvious: paying high prices for many dozens of single-treatment, lifetime cures – from which they likely will not see most of the promised clinical and economic benefits. How will they manage these new therapies – what kinds of restrictions will they – can they – impose? And what can or should biotechs do to mitigate these challenges?*

Speakers:

- **Moderator: Nick Calla**, SVP, Industry relations, Orsini Specialty
- **Paul Jeffrey**, former Chief Pharmacy Officer at MassHealth (Medicaid)
- **Chronis Manolis**, SVP, Pharmacy, UPMC Health Plan
- **Luke Prettol**, Lead Benefits Strategy Consultant, AT&T
- **Rob O'Brien**, SVP, Specialty, Real Endpoints

## 11:45 – 12:00 Group reflection and meeting close

## 12:00 Boxed lunches