



# OUR VISION FOR THE FUTURE OF Precision Genetic Medicine

7:30 a.m. – 7:50 a.m.

## Sarepta's Mission

DOUG INGRAM, President & Chief Executive Officer, Sarepta Therapeutics

7:50 a.m. – 8:10 a.m.

## Clinical Development Approach and Philosophy

GILMORE O'NEILL, M.B., M.M.SC., Chief Medical Officer, Sarepta Therapeutics

8:10 a.m. – 8:40 a.m.

## A Phase 1/2 Clinical Trial of Intra-arterial Gene Transfer of rAAVrh74.MCK. GALGT2 for Duchenne Muscular Dystrophy: Initial Safety Profile

KEVIN M. FLANIGAN, M.D., Director, Center for Gene Therapy, Nationwide Children's Hospital

8:40 a.m. – 9:10 a.m.

## Micro-dystrophin Gene Therapy Approach for the Treatment of Duchenne Muscular Dystrophy

SERGE BRAUN, PHARM.D., PH.D., Chief Scientific Officer, AFM-Téléthon;  
Director Neuromuscular Strategy, Genethon; President, Genosafe SAS;  
Member, French National Academy of Pharmacy

9:10 a.m. – 9:20 a.m.

## Break

9:20 a.m. – 10:20 a.m.

## Systemic Delivery of AAVrh74.MHCK7. Micro-dystrophin for Duchenne Muscular Dystrophy (Preliminary Results from Phase I/II Clinical Trial)

JERRY R. MENDELL, M.D., Curran-Peters Chair of Pediatric Research, Center for Gene Therapy,  
Nationwide Children's Hospital

LOUISE RODINO-KLAPAC, PH.D., Vice President, Gene Therapy, Sarepta Therapeutics;  
Chief Scientific Officer, Myonex Therapeutics

10:20 a.m. – 10:50 a.m.

## Genome Editing for Duchenne Muscular Dystrophy

CHARLES A. GERSBACH, PH.D., Associate Professor of Biomedical Engineering;  
Director, Center for Biomolecular and Tissue Engineering, Duke University

10:50 a.m. – 11:20 a.m.

## LGMD Gene Therapy

LOUISE RODINO-KLAPAC, PH.D., Vice President, Gene Therapy, Sarepta Therapeutics;  
Chief Scientific Officer, Myonex Therapeutics

11:20 a.m. – 11:50 a.m.

## Lunch Break

11:50 a.m. – 12:30 p.m.

## The Chemical Architecture of PPMO

GUNNAR J. HANSON, PH.D., Senior Director, Research Chemistry, Sarepta Therapeutics

## PPMO for Duchenne Muscular Dystrophy

MARCO PASSINI, PH.D., Senior Director, Biology, Sarepta Therapeutics

12:30 p.m. – 1:15 p.m.

## Q&A Session (All Speakers)

1:15 p.m. – 1:30 p.m.

## Closing Remarks

DOUG INGRAM, President & Chief Executive Officer, Sarepta Therapeutics