Featured Cell and Gene Therapy Thought Leadership:
Virtual ISPOR 2021 and More

VIRTUAL ISPOR 2021 PRESENTATIONS AND POSTERS:

The New SMC-Ultra-Orphan Pathway: HTA Best Practice for Very Rare Diseases? | This presentation shares challenges for payers and HTA best practices for evaluating medicines in ultra-orphan indications.

Presenter: Richard Macaulay
When: Prerelease session now available!

Global Access Implications of Germany's New GSAV Law for Orphan Drugs | This research evaluates the potential impact of the GSAV law through examining any orphan drug subject to a new benefit assessment, after exceeding the annual €50 million threshold.

Authors: Christina Poschen, Sabina Anwar, Richard Macaulay
When: Wednesday, May 19, 11:30 AM-1:45 PM EST

The Cost-Effectiveness of OTL-200 for the Treatment of Metachromatic Leukodystrophy (MLD) | This study determined the long-term cost-effectiveness of a recently approved ex vivo gene therapy (OTL-200) compared to best supportive care for the treatment of MLD.

Authors: Francis Pang, Rebecca Dean, Ivar Jensen, Andrew Olaye, Beck Miller
When: Wednesday, May 19, 11:30 AM-1:45 PM EST

An Observational Cohort Analysis on the Economic Impact of Chronic Kidney Disease (CKD) in Patients with Fabry Disease (FD) | In this study, health care resource utilization and related costs were evaluated to better understand the economic impact of CKD in patients with FD.

Authors: Eric Wallace, Naomi Sacks, Phil Cyr, Marissa Baker-Wagner
When: Wednesday, May 19, 11:30 AM-1:45 PM EST

RECENT SCIENTIFIC PUBLICATIONS

Costs and Health Resource Use in Patients with X-linked Myotubular Myopathy: Insights from U.S. Commercial Claims

Authors: Naomi C. Sacks, Bridget E. Healey, Phil Cyr, Theodore Slocomb, Emma James, Alan H. Beggs, Robert J Graham

An updated cost-utility model for onasemnogene abeparvovec (Zolgensma®) in spinal muscular atrophy type 1 patients and comparison with evaluation by the Institute for Clinical and Effectiveness Review (ICER)

Authors: Rebecca Dean, Ivar Jensen, Phil Cyr, Beckley Miller, Benit Maru, Douglas M. Sproule, Douglas E. Feltner, Thomas Wiesner, Daniel C. Malone, Matthias Bischof, Walter Toro, Omar Dabbous

GENE THERAPY FOR RARE DISORDERS

Check out Precision ADVANCE'S panel discussion at the 4th annual Gene Therapy for Rare Disorders Conference.

AMCP AND PRECISION VALUE & HEALTH CORPORATE TRAINING PROGRAM

Register for "Overcoming Unique Challenges in Evidence Development and Assessment for Access and Reimbursement/ Coverage of Cell and Gene Therapies," an AMCP and Precision Value & Health Corporate Training Program

Featuring: Precision's Larry Blandford, Phil Cyr, Erin Lopata

This half-day course will provide foundational information to equip the audience with how to best develop a compelling evidence package for cell and gene therapies, how payers will apply their assessment in decision-making, and how to best bridge the gap between these two.

Connect with one of our experts.
Contact us at **precisionadvance@precisionmedicinegrp.com**.

PRECISION ADVANCE the cell & gene therapy collective™

To learn more about Precision ADVANCE, visit **precisionmedicinegrp.com/advance**.