



Gene Therapies for Sickle Cell Disease: Effectiveness and Value

Thursday • July 27th, 2023 • 9:00 AM – 3:00 PM PT

Virtual Event – Zoom Webinar

https://us02web.zoom.us/webinar/register/WN_VcNbenSfQjCajIYSbgDetg

| Time (PT) | Activity |
|---------------------|--|
| 9:00 am – 9:20 am | Meeting Convened and Opening Remarks Steven D. Pearson, MD, MSc President, ICER |
| 9:20 am – 10:00 am | Presentation of the Clinical Evidence Francesca L. Beaudoin, MD, PhD, MS Senior Medical Advisor, ICER |
| 10:00 am – 10:40 am | Presentation of the Economic Model Praveen Thokala, PhD, MASc Senior Research Fellow, School of Health and Related Research, University of Sheffield |
| 10:40 am – 11:20 am | Public Comments and Discussion |
| 11:20 am – 12:05 pm | Lunch Break |
| 12:05 pm – 1:05 pm | CTAF Vote on Clinical Effectiveness and Value |
| 1:05 pm – 1:15 pm | Break |
| 1:15 pm – 2:45 pm | Policy Roundtable |
| 2:45 pm – 3:00 pm | Reflections from CTAF |
| 3:00 pm | Meeting Adjourned |

| ICER Staff and Consultants | |
|--|--|
| Francesca Beaudoin, MD, PhD, MS , Senior Medical Advisor, ICER* | Dmitriy Nikitin, MSPH , Senior Research Lead, Evidence Synthesis, ICER* |
| Jon Campbell, PhD, MS , Senior Vice President for Health Economics, ICER* | Becca Piltch, MPP , Program Associate, ICER* |
| Kelsey Gosselin, MA , Program Manager, ICER* | Steve Pearson, MD, MSc , President, ICER* |
| Avery McKenna, BS , Associate Research Lead, ICER* | David Rind, MD, MSc , Chief Medical Officer, ICER* |
| Emily Nhan, BA , Senior Research Assistant, ICER* | Praveen Thokala, MASc, PhD , Senior Research Fellow, University of Sheffield* |

*No conflicts of interest to disclose, defined as individual health care stock ownership in any health plan or pharmaceutical, biotechnology, or medical device manufacturers, or any health care consultant income or honoraria from health plans or manufacturers.

Participating Members of CTAF

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| Ralph Brindis, MD, MPH , Clinical Professor of Medicine, UCSF* | Joy Melnikow, MD , Professor Emeritus, University of California Davis* |
| Robert Collyar , Patient Advocate, Patient Advocates in Research* | Elizabeth Murphy, MD, Dphil , Professor of Medicine, UCSF* |
| Rena Fox, MD , Professor of Medicine, UCSF* | Kathryn Phillips, PhD , Professor, UCSF* |
| Kimberly Gregory, MD, MPH , Vice Chair OB/GYN, Cedars Sinai Medical Center* | Ann Raldow, MD, MPH , Associate Professor, UCLA* |
| Paul Heidenreich, MD, MS , Professor, Stanford University School of Medicine* | Rita Redberg, MD , Professor of Medicine, UCSF* |
| Jeffrey Hoch, MA, PhD , Professor, University of California Davis* | Alex Smith, MD, MS, MPH , Professor of Medicine, UCSF* |
| Jeff Klingman, MD , Chair of Neurology, Kaiser Permanente NCAL* | Joanna Smith, LCSW, MPH , CEO, Healthcare Liaison* |

*No conflicts of interest to disclose, defined as individual health care stock ownership (including anyone in the member's household) in any company with a product under study, including comparators, at the meeting in excess of \$10,000 during the previous year, or any health care consultancy income from the manufacturer of the product or comparators being evaluated.

| Policy Roundtable Participant | Conflict of Interest |
|--|---|
| Cecelia Calhoun, MD, MPHS, MBA | Nothing to disclose. |
| Jaime Rubin Cahill, MA, MPH | Jaime Rubin Cahill is a full-time employee of Vertex Pharmaceuticals. |
| Elle Cole, BA | Nothing to disclose. |
| Michelle Gourdine, MD | Dr. Gourdine is an employee of CVS Health. She receives equity interest in excess of \$10,000 Agilon Health, had status as a Agilon Health board director, as a Horizon BCBS NJ board director, and at the University of Maryland Medical System. |
| Jimi Olaghery | Nothing to disclose. |
| Patrick McGann, MD, PhD | Dr. McGann received monetary value in excess of \$5,000 after serving on a Novartis Safety Advisory Board. |
| Clark Paramore, MSPH | Clark Paramore is a full-time employee of bluebird bio |
| John Watkins, PharmD, MPH, BCPS | Nothing to disclose. |