



ELSI FRIDAY FORUM

Value and Values in Payment for Gene Therapies

Date and time: Friday, May 12, 2023 | 12pm ET US / 9am PT US

Speakers: Renske M.T. ten Ham, PhD, PharmD, MSc & R. Brett McQueen, PhD

Moderator: Hadley S. Smith, PhD, MPSA

Biographies

Moderator: Hadley S. Smith, PhD, MPSA

Biography: <https://www.populationmedicine.org/hsmith>

Panelist: Renske M.T. ten Ham, PhD, PharmD, MSc

Biography: <https://www.umcutrecht.nl/en/research/researchers/ten-ham-renske-rmt>

Panelist: R. Brett McQueen, PhD

Biography: <https://pharmacy.cuanschutz.edu/about-us/profile/brett-mcqueen>

ELSIhub Collections

ELSIhub Collections are essential reading lists on fundamental or emerging topics in ELSI, curated and explained by expert collection editors. Please use the link to access Paying for Cures: The Ethics and Economics of Gene Therapies for Rare Diseases curated by Meghan Halley, PhD, MPH, Senior Research Scholar, Center for Biomedical Ethics, Stanford University School of Medicine.

<https://elsihub.org/collection/paying-cures-ethics-and-economics-gene-therapies-rare-diseases>

Engaging Underrepresented Communities in Research, a CERA interview by Deanne Dunbar Dolan and Rachel H. Lee:

<https://elsihub.org/news/engaging-underrepresented-communities-research>

Hadley S. Smith, PhD, MPSA



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Link to “2022-2024 Working Group: Value Ethics: Mapping Strategies for Value Assessment and Reimbursement of Gene Therapies for Rare Diseases”:

<https://elsihub.org/working-group/2022-2024-working-group/value-ethics-mapping-strategies-value-assessment-and>

Renske M.T. ten Ham, PhD, PharmD, MSc

Link to “EUnetHTA HTA Core Model User Guide” (2016):

https://www.eunetha.eu/wp-content/uploads/2018/06/HTACoreModel_UserGuide_Version1.1-1.pdf?x50316

Link to “Europe’s patchwork of evidence requirements is an important factor in delayed patient access”:

<https://www.vintura.com/news/europe-evidence-requirements-important-factor-in-delayed-patient-access/>

Link to “Reported Challenges in Health Technology Assessment of Complex Health Technologies” (2022): <https://doi.org/10.1016/j.jval.2021.11.1356>

R.M.T.tenham-2@umcutrecht.nl

R. Brett McQueen, PhD

Link to “What principles should govern the use of managed entry agreements?” (2011):

<http://dx.doi.org/10.1017/S0266462310001297>

Link to “Performance-based managed entry agreements for new medicines in OECD countries and EU member states: How they work and possible improvements going forward” (2019):

<https://dx.doi.org/10.1787/6e5e4c0f-en>



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Link to “Performance-Based Risk-Sharing Arrangements: An Updated International Review” (2017): <https://doi.org/10.1007/s40273-017-0535-z>

Link to FoCUS: <https://newdigs.tuftsmedicalcenter.org/programs-projects/focus/>

Link to “Establishing the Prevalence and Prevalence at Birth of Hemophilia in Males” (2019): <https://doi.org/10.7326/M19-1208>

Link to “The epidemiology of inhibitors in haemophilia A: a systematic review” (2003): <https://doi.org/10.1046/j.1365-2516.2003.00780.x>

Link to “Inhibitor incidence in an unselected cohort of previously untreated patients with severe haemophilia B: a PedNet study” (2021): <https://doi.org/10.3324/haematol.2019.239160>

Link to “A Report in Response to the Executive Order on Lowering Prescription Drug Costs for Americans” (2023): <https://innovation.cms.gov/data-and-reports/2023/eo-rx-drug-cost-response-report>