



THE UNIVERSITY OF ARIZONA HEALTH SCIENCES

Center for Applied Genetics & Genomic Medicine

PRESENTS

Genetics and Genomics Grand Rounds September 19, 2018 | 4 – 5pm | MRB Room 102

- CME CREDIT AVAILABLE -
- REFRESHMENTS PROVIDED -

For more information, please visit

<http://precisionhealth.uahs.arizona.edu/genetics-genomics-grand-rounds>

Livestream available at <https://goo.gl/E5AR2F>.

Contact Barbara Kahn-Sales | bkahnsales@email.arizona.edu

JESSE'S INTENT: THE IMPORTANCE OF INFORMED CONSENT & CLINICAL TRIAL OVERSIGHT

At the age of two, Paul Gelsinger's son, Jesse, was diagnosed with ornithine transcarbamylase (OTC) deficiency, a rare genetic condition that causes ammonia to build up in the bloodstream. Thus began a life-changing journey for the whole family. In September 1999, 18-year-old Jesse Gelsinger traveled from his home in Tucson to participate in a clinical trial at the University of Pennsylvania. Jesse died after receiving an experimental gene therapy-based treatment in a phase 1 clinical trial for OTC deficiency.

To honor his son's memory, Paul Gelsinger became a patient advocate, working since 2000 to change the gene-therapy research culture. He has given numerous presentations about his family's experience to a wide range of audiences, including at many medical centers, legal and ethics conferences, and also testifying before Congress. He has contributed to educational materials about OTC deficiency for the National Organization for Rare Disorders, and authored a publication describing his son's experience. To reach a broader audience, he participated in documentaries about gene therapy clinical trials, and continues to advocate for strengthening informed consent and human subject research protections.

Mr. Gelsinger will touch on his personal experiences, and review the evolution of gene therapy, the real and potential conflicts of interest in clinical research, and the awareness of these issues – or not – by research participants.



Paul L. Gelsinger

*Parent and Patient
Advocate*