Advanced Therapies Medicinal Products (ATMPs) CoP

ATMPs CoP Representatives

- Erich Bozenhardt
  - CoP SC Chair
  - Assoc. Director Process Engineering, United Therapeutics, North Carolina, USA
- Zhimei Du
  - CoP SC Member
  - Vice President, Translational Research & Early Development, Landmark Bio, Massachusetts, USA
- Nessiem Samuel
  - CoP SC Member
  - Founder and Partner, GxP Impact Consulting LLC, AZ, CA, CO, IN, MA, MD, NC, NJ, PA, SC, TN, VA, TX, USA
- Biana Torres
  - CoP SC Member
  - Head of Quality, Theragent, California, USA

ATMPs CoP Focus

- Focus on the unique elements and challenges in ATMP (cell and gene therapies) for both autologous and allogenic therapies.
- Manufacturing (from collection/banking to patient) processes/techniques that reduce cost of goods with consistent product quality. including The development of a robust control framework to ensure product quality, including CMC/GMP and unique equipment challenges.
- Best practice analytical and stability methodologies, and strategies for raw materials, In process, and accelerated product release.
- Regulatory landscape and challenges - Identifying and determining solutions for complex regulatory issues that, when resolved, will result in accelerated speed to market.
- Phase appropriate CMC development strategy to improve the success rate of cell/gene therapy manufacturing and regulatory filings in both early and late development stages.

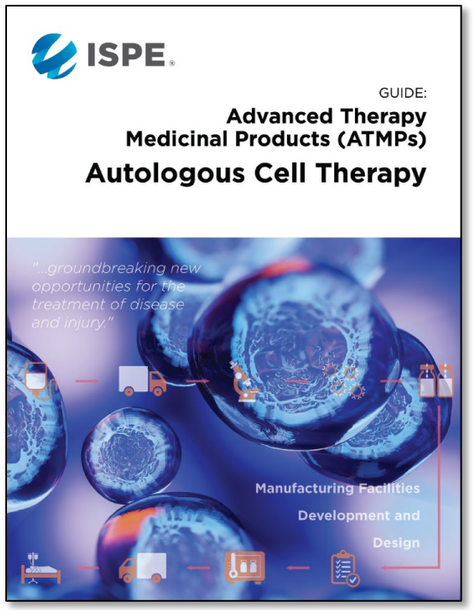
ATMPs CoP Recent Activities

ISPE Guides

- ATMPs - Autologous Cell Therapy (published 2021)
- AAV Comparability and Lifecycle Management (published 2023)
- ATMPs - Allogeneic Cell Therapies (published 2023)
- ATMP Phase Appropriate/Risk Based Validation (planned 2024)
- ATMPs - Cell Therapy Equipment Design (planned 2025)

Conference Presentations

- Aseptic Conference
- Biotechnology Conference
- Europe Conference
- Annual Meeting & Expo



Guides

- Cell Therapy Equipment Design
  - Charles Heffernan, charles.heffernan@pmgroup-global.com
  - Co-lead: Tony Thatcher anthony.thatcher@us.sumitomo-pharma.com
  - Revise to lessen commerical aspect
  - Industry review target mid Aug(?)
- ATMP Risked Based Validation
  - Biana Torres, Lead, bianaytorres@gmail.com
  - Co-lead: Jean Duliere, jfd@duliere.eu
  - Industry review target mid year
- Non Viral Gene Therapy Process Development
  - Emily Heffernan, emily.heffernan@arcadis.com
  - Nidhi Shah nidhi.shah@bms.com
  - Robert Dream, robert.f.dream@gmail.com
  - Developing scope document
  - RNA facility guide scope approved, this guide is the companion that covers process

Other activities

- *Upcoming*
  - *2024 Annual several submitted*
    - *Feedback completed*
  - *2024 Bio*
    - *Judith to lead ATMP track*
  - *Boston Products Show*
    - *2 Oct*
    - *Nessiem*
  - *2024 Annex 1 / Pharma 4.0*
    - *Judith and digital solutions for ATMP working group*
  - *2025 Aspetic*
    - *Need to develop ATMP track*
    - *Erich and Nidhi Shah leading track*
    - *Open for proposals (closes 29 July)*

Annual Meeting

- CoP meeting Wednesday 16 Oct 14:15-15:15pm EST
  - Emerald 4 and virtual
- Phase & Risk Based Approaches to Enhance ATMP Validation
  - Biana & Ryan from guide team
- Allogeneic Cell Therapy
  - Komal & Ian from guide team
- CoGs
  - Autologous: comparing open, closed, isolated, automated & isolated
  - Workshop & 30min presentation
  - Combined with similar proposal by Jeff Odum
- Translational Technologies and Precision Medicine
  - Delivery systems LNPs / encapsulation of cells?
  - Tissue specific gene therapy delivery? –George might be able to help
  - Co-present with Combination products CoP
  - Need to confirm speaker
- CMC / Regulatory
  - Krisha and Stephine Martin

Thank you!

Questions