VERISTAT

Roadmap to Market for Cell and Gene Therapies

Development of cell and gene therapies is complicated, with every product presenting a unique set of challenges. It's critical to have an experienced partner to help you engage with regulators and design the study early on. Having executed trials for the first gene therapy approved in Europe, Veristat set the standard for other therapies in development and remains a leader in this specialized area today. With our global team of scientific experts offering strategic insights and custom-tailored solutions, Veristat provides comprehensive end-to-end services, from pre-IND to submissions and approvals.

ADVANCING THESE COMPLEX PROJECTS THROUGH THE ENTIRE DEVELOPMENT PATHWAY

PLANNING – Get it right from the start

- Regulatory consulting, approvals, publishing
- Initial consulting and strategic regulatory assessments – especially important for C&G programs to get interactions with FDA and EMA early
- Scientific Advice meetings
- Parallel EMA-FDA scientific advice for breakthrough drugs
- Acting as official agency representative
- Pre-clinical and clinical development strategic consulting
- CMC support
- Qualification of novel methodologies

US

- Interact meetings with FDA
- Pre-IND meetings with FDA to define agency expectations and get all questions answered
- Engagement with FDA's Emerging Technologies Program – outlining novel technologies and manufacturing approaches with which the agency is less familiar
- Plan for RMAT and/or orphan drug application
- Plan for breakthrough or fast-track designations

EU

- Certification of Advanced Therapy Medicinal Products (ATMP)
- EMA CAT categorization
- Protocol assistance for orphan drugs
- Joint feedback from regulators and HTA bodies
- Paediatric Investigation Plan/ deferral or waiver requests
- Innovation task force (ITF)
- PRIME designation
- Orphan designation
- SME designation

EXECUTION – Flexible, on-time, done right

- Site startup
- Patient recruitment
- Monitoring
- Data Management
- Project Management
- Medical Affairs
- Medical Writing
- Biostats
- Full service regulatory dossier management (authoring, publishing & electronic submission)
- Continued support for regulatory communications and IND/CTA amendments
- Natural history studies
- *Ex vivo* (via autologous stem cells and allogeneic stem cells)
- Retroviral and lentiviral vector-mediated gene therapy
- Centralized site model
- Remote monitoring
- SMC / DMC / DSMB
- Long-term follow-up studies for interventional study rollover from Phase I/II

US

- Regulatory support for additional FDA meetings – Type C, End of Phase II, Pre-BLA meetings
- US Agent/US Authorized Legal Representative
- IND maintenance (Annual Regulatory/Safety Reporting, Information Amendments)

EU

- Certification procedure of quality and non-clinical data
- Regulatory support for additional scientific advice meetings as clinical development progresses, continued engagement of regulatory authorities
- EU Legal Representative
- Annual Regulatory/ Safety Reporting

SUBMISSION – Proven track record of success

- Initial Marketing Applications (authoring, publishing, submission)
- Additional Marketing Applications in other regions e.g. Japan FDA (authoring, publishing, submission)
- Support of inspection readiness activities in preparation for MA regulatory inspection

US

- NDA and BLA applications (Authoring, Publishing, Submission)
- Gap analysis of NDA/BLA Modules
- Regulatory support for NDA/BLA early, mid- and late cycle review meetings during review of licensing applications
- Supporting inspection-readiness activities with external training of Sponsor, vendor and/or clinical sites tailored to FDA
- Continued post-approval regulatory support

EU

- EMA Marketing Authorization Applications (MAA) (authoring, publishing, submission)
- Gap Analysis of MAA Modules
- Supporting inspection-readiness activities with external training of Sponsor, vendor and/or clinical sites tailored to EMA
- Continued support following Full, Conditional and/or Accelerated approvals

POST-MARKETING – Realizing a therapy's full potential

- Management of Phase IV studies
- Ongoing support and/or management for long-term follow-up studies and post-marketing commitments
- Pharmacovigilance and safety monitoring
- Payer and reimbursement submissions
- Post-marketing study commitments (PASS, REMS)

EXPERIENCE IN ALL MAJOR CATEGORIES OF CELL AND GENE THERAPY

Cell therapy

The injection, grafting or implantation of viable autologous, allogeneic or xenogeneic cells into the patient

Gene therapy

Gene addition or editing (disruption, inactivation, correction or insertion) either *in vivo* or *ex vivo*

Tissue therapy

Cells or tissues modified for repair, regeneration or replacement

Real-world examples from every stage of development

Planning for a novel cell therapy product

Induced pluripotent stem cell (iPSC)-based therapy administered in humans only in two other indications around the world, never before used in a neurological indication. Provided key pre-clinical, CMC and clinical development plan consulting, supported client in preparation, attendance and response for EU Scientific Advice Meeting, obtaining successful, positive Competent Authority buy-in. Continued support in EU CTA preparation and planned full service management of the Phase I trial.

Execution of a gene therapy program

Full-service support of a rare disease gene therapy program from Phase I/II to Phase III in the US and EU. Proactive, flexible support throughout the program delivery enabling prompt restructuring of full-service support in response to mid-program changes, following End of Phase II/pre-BLA FDA meetings and client commercial re-focusing.

Filing new marketing application/components of an EMA MAA

Successfully supported the EMA MAA submission and approval of the first gene therapy to be approved in Europe following support on EU and North American studies, including the long-term follow-up. Generated combined SDTM/ADaM compliant MAA datasets; prepared SAE/patient narratives and CSRs for inclusion in MAA Module 5; prepared MAA Module 2 and Integrated Summary of Efficacy (ISE)/Integrated Summary of Safety (ISS) for FDA submission.

Delivering on post-market commitments

Provided regulatory, project management, site management and monitoring services for a post-EMA approval PASS commitment for a retrospective retroviral insertion site analysis of stored blood samples for gene therapy treated clinical trial and commercial patients, successfully ensuring ongoing MAA approval. Provided full service for 15-year long-term follow-up studies enrolling clinical trial patients treated with gene therapy, enabling ongoing safety monitoring.

The Cell and Gene Therapy Partner of Choice

We understand how high the stakes are with your cell and gene therapy program. We know that nothing is standard about study design, study conduct or regulatory process in this specialized area. Veristat has successfully supported more than 100 cell and gene therapy projects, with a seasoned global team of scientific experts who are adept at strategy and execution across the clinical development pathway.

CONTACT VERISTAT

www.veristat.com