Cell and Gene Therapy Product Development Matrix – Clinical

	Optimization (Research up to Pre-IND)	Development (Pre-IND to IND)
Clinical Trial Design References 1, 2, 3, 4, 5	• Description of proposed clinical application(s), unmet need. • Therapeutic rationale for the proposed investigational product. o Hypothesized mechanism of action/biological function(s) • The Pre-IND briefing document should include an outline of the proposed clinical trial, specifying: o Study description and rationale o Target indication o Subject population, sample size o Dosing levels and regimen, cohort design o Route of product administration o Dosing procedures o Outcome measures, parameters to be assessed o Long-term clinical monitoring program (for gene therapy products, should include rationale for monitoring duration, typically 15-30 years)	• As for Optimization but more detailed and should address relevant FDA comments from Pre-IND meeting.
Biostatistics	• Letter of support from biostatistician. • Draft description of statistical analysis plan.	• Letter of support from biostatistician. • Statistical analysis plan
Institutional Review Board/Human Subjects Committee	N/A	Status, scored from A (highest) to D (lowest) A. IRB approval B. IRB application, informed consent in revision C. IRB application submitted D. IRB application, informed consent document in preparation
Recombinant Advisory Committee (required for certain gene therapy products)	N/A	If RAC review is required: Status, scored from A (highest) to C (lowest) A. RAC approval B. RAC review completed C. RAC application in preparation

References

Reference	Title	Description	File Name
1	FDA Guidance for Industry: Considerations for the Design of Early-Phase Clinical Trials of Cellular and Gene Therapy Products	Regulatory considerations and requirements for clinical trial design in cell therapy and gene therapy.	FDA Guidance - Considerations for the Design of Early-Phase Clinical Trials of Cellular and Gene Therapy Products.pdf
2	FDA Guidance for Industry: Gene Therapy Clinical Trials - Observing Subjects for Delayed Adverse Events	Regulatory requirements for long-term monitoring in gene therapy clinical trials.	FDA Guidance - Gene Therapy Clinical Trials, Observing Subjects for Delayed Adverse Events.pdf
3	PAS 83: Developing human cells for clinical applications in the EU and USA - 2012	Overview of cell therapy product development, including practical aspects and US FDA regulatory considerations.	PAS 83 - Developing human cells for clinical applications in the EU and USA – 2012.pdf
4	USP <1046> Cellular and Tissue-based Products	USP chapter on development of cell therapy and tissue-engineered products, emphasis on CMC aspects but addresses clinical as well.	USP 1046 - Cellular and Tissue-based Products (NF, Supplement).pdf
5	USP <1047> Gene Therapy Products	USP chapter on development of gene therapy products, emphasis on CMC aspects but addresses clinical as well.	USP 1047 - Gene Therapy Products (NF, supplement).pdf