



2019 ASGCT Policy Summit
Global Regulatory Issues in Gene Therapy Development
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From early studies to exponential growth

First gene therapy trial
in US at NIH for ADA
deficiency inspires early
clinical research
(1990)



1990

Vector science
advances and
promising vectors
emerge



Exponential
growth in gene
therapy research
and development

Today

Today: Our shared challenge



Unmet needs of patients
inspire collaboration and
swift action



Gene therapy science and
technology has outpaced
evolution of our regulatory
frameworks

Looking forward



Multi-stakeholder collaboration – learning together through experience to evolve policy



Continued development and implementation of pragmatic regulatory policies for gene therapy development and expedited review



Continued innovation to align development strategies and regulatory expectations with patient needs