

Future Directions and Resource Needs for NHLBI Gene Therapy Research
Virtual Meeting Format

March 15-16, 2022

DAY 1- Tuesday, March 15, 2022

12:00 pm-4:45 pm EST

12:00 pm-12:10 pm	<p>Welcoming Remarks and Meeting Overview</p> <ul style="list-style-type: none"> Welcome: DCCS Division Director, Dr. Goff or other- 5 minutes Overview and Goals: Workshop Chair, Dr. Terry Flotte-5 minutes
12:10 pm-12:30 pm	<p>GTRP History and Impact</p> <ul style="list-style-type: none"> History of the GTRP: NHLBI Staff-5-10 minutes Evolution of the Field of Gene Therapy and the GTRP: Don Kohn 10-15 minutes
	<p>Session 1:</p> <p align="center">Big Picture View of the Current State of Gene Therapy and Gene Editing</p>
12:30 pm-1:00 pm	Jim Wilson and Fyodor Urnov
1:00 pm-1:10 pm	Open Discussion
	<p>Session 2:</p> <p align="center">Emerging Technologies and Future Directions</p> <p>Moderators: Fyodor Urnov and Matt Porteus</p>
	<p>Gene Therapy & Editing for Specific Indications</p>
1:10 pm-1:30 pm	David Liu
1:30 pm-1:50 pm	David Rawlings
1:50 pm-2:10 pm	Shengdar Tsai
	<p>The Status of DNA Cutting, Delivery Modes, and Molecular Biology Applications</p>
2:10 pm-2:25 pm	BREAK
2:25 pm-2:45 pm	Michael Mitchell
2:45 pm-3:05 pm	Gang Bao

	<i>Breakout Groups to Discuss the Resource Needs for Advancing the Emerging Technologies</i>
3:05 pm-4:05 pm	Two breakout groups discuss the future research directions and facilitating resources guided by specific questions
4:05 pm-4:35 pm	Reports from each breakout group (15 minutes each)
4:35 pm-4:45 pm	Summary of the day and adjournment until tomorrow
DAY 2- Wednesday, March 16, 2022	
12:00 pm-5:10 pm EST	
12:00 pm-12:10 pm	Welcome to Day 2 of the Workshop Terry Flotte
	Session 3: Challenges in Clinical Translation and Implementation Moderators: <i>Punam Malik and Terry Flotte</i>
	<i>Challenges and Lessons Learned in Moving from Bench to Bedside</i>
12:10 pm-12:25 pm	Barry Byrne
12:25 pm-12:40 pm	William Pu
	<i>Challenges and Lessons Learned in Funding/Conducting Clinical Trials and Beyond</i>
12:40 pm-12:55 pm	Punam Malik
12:55 pm-1:10 pm	Hans Peter Kiem
1:10 pm-1:25 pm	David Williams
1:25 pm-1:40 pm	Richard Colvin
1:40 pm-2:10 pm	BREAK
	Session 4: From There to Here, and Where the Field is Going: Identifying Research Gaps, Opportunities, and Resource Needs Moderators: <i>Fyodor Urnov, and Terry Flotte</i>
	<i>From There to Here, and Where the Field is Going</i>
2:10 pm-2:30 pm	David Schaffer
2:30 pm-2:50 pm	Dan Anderson
2:50 pm-3:30 pm	Jim Wilson - Summary of the past and the future of gene therapies

	<i>Identifying Research Gaps, Opportunities, and Resource Needs</i>
3:30 pm-4:10 pm	Two breakout groups discuss the research gaps, opportunities, and resource needs guided by specific questions
4:10 pm-4:40 pm	Report from each breakout group (15 minutes each)
	Session 5: Summary Session
4:40 pm-5:10 pm	<u>Overall Summary of Key Workshop Discussion Points and Next Steps</u> Terry Flotte, Matt Porteus, and All Participants
5:10 pm	Workshop Concludes