

Draft schedule — FUS GCT Workshop

Monday Morning

	Intro Session
8:00-8:15	Welcome & Workshop Intro (Fred)
8:15-8:45	Who's Doing What — Intros
8:45-9:00	FUSF perspective: What Programs to Support? — Alec Batts (UVa & FUSF)
	Session 1: Overview presentations Moderator:
9-9:30	Large Organ GT: Challenges/Opportunities, recent FUS Data — Jonathan Lindner (UVa)
9:30-10	FUS for Brain GT: Challenges, Opportunities, recent FUS data — Elisa Konofagou (Columbia University)
10-10:30	Break
	Session 2: Presentations Moderator: Elisa Konofagou
10:30-12	<ul style="list-style-type: none"> - Thermogenetic control of CAR T cells to potentiate brain tumor therapy, Lena Gamboa (Georgia Tech) - AAV capsid engineering for liver detargeting and redirecting cellular and tissue tropism, Jared Smith (RegenXBio) - FUS-mediated gene replacement therapy and gene editing for the treatment of hemophilia, Carol Miao (Seattle Children's Research Institute) - Dissecting Routes to the Brain: CSF administration vs. systemic AAV delivery in NHPs, Michal Fortuna (Allen Institute on Brain Research)
12-1	Lunch

Monday Afternoon

	Session 3: Overview presentations Moderator:
1-1:30	Cell therapy of brain tumors — Catherine Bollard (Children's National, DC)
1:30-2	Routes of administration & Platform Approach for GT — Bryan Pukenas (UPenn)
	Session 4: Breakouts & Cross-Pollination Moderator:
2-2:30	<p>Flash talks</p> <ul style="list-style-type: none"> - Delivery of ASO for rare pediatric neurodegenerative disease, Laura Owlett (University of Rochester) - Noninvasive focal gene transfer of chemogenetic proteins in the primate brain, David Schaeffer (U Pittsburg) - FUS-mediated for cell therapy of brain tumors, Dalia Haydar (Children's National) <p>Breakout Instructions — Fred (5 min) 7</p>
2:30-3:15	<p>Breakout Round 1</p> <p>1A: From small animals to primates to humans 1B. FUS for brain cell therapy 1C. Routes of administration for FUS + GCT</p>
3:15-4	<p>Breakout Round 2</p> <p>2A: Ideal FUS system characteristics for brain & large organ GCT 2B. Large organs GCT 2C. Overcoming biological & technical barriers to FUS + GCT</p>
4-4:30	Break (and group photo!)
4:30-5:30	Breakout Reporting & Open discussion
6:30	Diner

Tuesday Morning

	Session 5: Presentations Moderator:
8-8:30	- Novel non-viral DNA delivery system , Jeff Bartlett (Rampart Bio) - FUS-mediated delivery of non-viral gene editing and gene therapy in the brain , Rich Price (UVa)
	Session 6: Regulatory Moderator:
8:30-9	Regulatory development in GCT - Michael Werner (Holland & Knight, ARM)
9-9:45	Panel discussion: What regulatory pathways for FUS GCT? <i>Panelists: Michael Werner, Jared Smith, Jon Lindner, Bryan Pukenas</i>
10-10:30	Break
	Session 7: Program Selection Moderator:
10:15-10:45	Current clinical trials and vector usage in gene therapy — Michael Lehmicke (ARM)
10:45-11:30	Panel & Discussion: How to select a program for FUS + GCT? <i>Panelists: Joe Bruder, Jennifer Johnston, Michael Kaplitt, Jon Lindner, Nathan Yozniak, Bob Smith</i>
11:30-12	Closing Discussion
Noon	Lunch to go