





August 1-3, 2019, Pullman Sydney Hyde Park, 36 College St, Sydney NSW 2010, Australia















































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Speaker's Note

All slide materials must be delivered in final form no later than 7:30 the day off the presentation to the presenter prep room via flash drive. If you would like to send your presentation in advance, please email to josh.thompson@thecurestartsnow. org.

CSN Medical Advisory Council

Gavin Baumgardner, Chair Nicholas Gottardo Alberto Broniscer Cynthia Hawkins Shana Coley
Daniel McPherson

Maryam Fouladi Michelle Monje-Deisseroth Stewart Goldman Roger Packer

Ancillary Meeting Rooms

Presentation Preparation Green Room - Busby Room, Second Floor
Ancillary Meeting Room - Dalley Room, Second Floor
MAC Meeting Room - Park View Room, Third Floor

Attendee Legend			
Open Attendance	Family and Foundations		
Doctors and Collaborative Sponsors	CSN Chapters		
Collaborative Sponsors	Special Ticket		

SESSIONS MARKED WITH "W" MAY BE AVAILABLE FOR WEBINAR VIEWING

Thursday, August 1	Windows on the Park - Pullman	Alternate Location
12:00-13:00		
13:00-14:00		Tour of Children's Cancer Institute - Shuttle provided from Pullman lobby,
14:00-15:00	International DIPG Registry Steering	TBA (Advance Registration Required)
15:00-16:00	Council Meeting (Invitation Only)	
16:00-17:00		
17:00-20:00	The Cure Starts Now Chapter Dinner	Hospitality Terrace Open - Terrace, Second Floor Pullman (open to families of attendees)
20:00-22:00	and Awards (CSN Chapters Only)	

Friday, August 2	lbis Room - Pullman	Alternate Location
7:30-8:00	Sponsored by BREAKFAST SERVED IN LOBBY	
8:00-8:20	Welcome and Introductions Keith Desserich, DIPG Collaborative and The Cure Starts Now Foundation; Ren Pedersen, The Cure Starts Now Foundation	
18.7()-8.4()	Review of Current Funded Research Results and Progress (Moderated by Gavin Baumgardner, The Cure Starts Now Foundation)	
	Repurposing Mefloquine for Diffuse Intrinsic Pontine Glioma Therapy – Bakhos Tannous, Massachusetts General Hospital	
8:40-8:55	Determine the Efficacy of BMP and MEK Inhibitors with RT to Treat ACVR1 Mutant DIPG — Christine Hoeman, Northwestern University	

8:55-9:10	Validation of TAK228 as an Effective Drug for Treating Children with DIPG – Javad Nazarian, Children's National Medical Center, George Washington University, Universität-Kinderspital)
9:10-9:25	Developing Novel Combination Therapeutic Approaches for DIPG Targeting Polo-like Kinase 1 – Laura Franshaw, Children's Cancer Institute (AU)	
9:25-9:40	Toward a Multimodality Cure for DIPG: Investigation of Intratumora Drug Penetration and Craniospinal Irradiation – Adam Green. Anchu Medical Campus	
9:40-9:55	Targeting Neuronal Activity Regulated DIPG Infiltration – Michelle Monje, Stanford University	
9:55-10:10	BREAK	
10:10-10:25	Targeting DIPG Through Pharmacological Activation of Mitochondria Biogenesis: an In Vitro and In Vivo Preclinical Study – Cynthia Hawkii The Hospital of Sick Children	
10:25-10:40	MRI Guided Focused Ultrasound: Towards Clinical Translation in DIP Saira Alli, The Hospital for Sick Children	PG -
10:40-10:55	Targeting EZH2 as a Therapeutic Strategy in DIPG – Richard Phillips, Memorial Sloan Kettering Cancer Center	
10:55-11:10	Investigating the Role of DIPG-derived Exosomes in Tumor Growth a Invasion – Maria Vinci, Ospedale Pediatrico Bambino Gesu	and
11:10-11:25	Elucidating the Underlying Mechanisms of Radio Resistance at Diagnosis and Relapse in DIPG – David Castel, Gustave Roussy	

11:25-11:40		Targeting DIPG through Combining a Super-activator (MCB-613) of Steroid Receptor Co-activators with Radiation – Xiao-Nan Li, Northwestern University
11:40-11:55	An Open Label Single Arm Phase I/II Study of MTX110 Delivered by CED in Patients with DIPG Previously Treated with External Beam Radiation Therapy - Sabine Mueller, The Regents of the University of California	
11:55-12:10		Combinational Strategies Alongside ACVR1 Inhibition in DIPG - Chris Jones, Institute of Cancer Research
12:10-12:25	LUNCH AVAILABLE IN LOBBY (Continues into next session)	
12:25-13:00	WORKING LUNCH - Cure Fund Grant (Moderated by Gavin Baumgardner) Presentation of 6 prospective novel research protocols (4 minutes each) with funding of \$50,000 to up to 2 winners as decided by the Medical Advisory Council.	
Sponsored by SNAPGRANT the uncommen, common app for grant research	12:27 Cure Fund Candidate – TBA	
	12:32	Cure Fund Candidate – TBA
	12:37	Cure Fund Candidate – TBA

	12:42	Cure Fund Candidate — TBA
	12:47	Cure Fund Candidate – TBA
	12:52	Summary of Cure Fund Presentations
13:00-13:20		Current Funded Research Results and Progress - CONTINUED ed by Gavin Baumgardner)
		Defining the Molecular Mechanisms of DIPG Development and Progression to Uncover Novel Therapeutic Targets - Jerome Fortin, The Campbell Family Institute for Cancer Research
13:20-13:35		Investigating the Role of DIPG-derived Exosomes in Tumor Growth and Invasion - Maria Vinci, Bambino Gesu Children's Hospital
13:35-13:50		A Mouse Model of HIST1H3B/ACVR1 Mutant DIPG - Chris Jones, The Institute of Cancer Research
13:50-14:05		Short-pulse Ultrasound Delivery of Panobinostat for the Treatment of DIPG in Children - Gail ter Haar, The Institute of Cancer Research
14:05-14:20		Credentialing an Improved DIPG Mouse Model - Oren Becher, Ann & Robert H. Lurie Children's Hospital
14:20-14:35		Imaging to Discern Immunotherapeutic-Induced Pseudoprogression in CNS Tumors - Kellie Haworth, St. Jude Children's Research Hospital

14:35-14:50		Polyamine Pathway Metabolism as a Novel Therapeutic Option for DIPG - Maria Tsoli, Sydney Children's Hospital
14:50-15:00		Summary – Gavin Baumgardner, Chair Medical Advisory Council, The Cure Starts Now
15:00-15:15	Review of	Cure Fund Grants (Moderated by Gavin Baumgardner)
		Using Zebrafish to Accelerate DIPG Drug Development – Eric Raabe, Johns Hopkins
15:15-15:25		Cellular Cybercriminals: DIPG Hacks into the Neural Communication Infrastructure – Michelle Monje, Stanford University
15:25-15:35		Advanced RNAi therapeutics for DIPG – Miguel Sena-Esteves, University of Massachusetts Medical School
15:35-15:50	BREAK	
15:50-16:05	Review of DIPG Data Collection Efforts (Moderated by Maraym Fouladi, International DIPG Registry)	
		Cavatica Update - Javad Nazarian, George Washington University, Universität-Kinderspital; Adam Resnick, Children's Hospital of Philadelphia
16:05-16:20		SIOPe DIPG Registry Update - Dannis van Vuurden, Princess Máxima Center for Pediatric Oncology
16:20-16:35		DIPG International Registry Update - Maryam Fouladi, Cincinnati Children's Hospital Medical Center

16:35-16:50	Connect Collaborative – Maryam Fouladi, Cincinnati Children's Hospital Medical Center	
116.50-17.20	New Institutional Member Forum for International Registry - Maryam Fouladi, Cincinnati Children's Hospital Medical Center	
17:20-17:30		
17:30-21:30		Networking Dinner — The Studio - reservation only, space limited (Presentation of Cure Fund Recipients, Collaborative Researcher of the Year and Research Innovator of the Year at 18:30) - short walk with directions

Saturday, August 3	Ibis Room - Pullman	Cook Room - Pullman
6:45-7:15	BREAKFAST SERVED IN LOBBY	
7:15-7:45		Collaborative Organizational Session - Heidi Feyerherm-Smith, The Cure Starts Now; Patrick Dunion, Aidan's Avengers; Steve Healey, Brooke Healey Foundation; Barrie Littlefield, Brain Cancer Advocate
7:45-8:15		New Member Forum - Seve Healey, Brooke Healey Foundation
8:15-8:45		Introduction to the DIPG Symposium - Barrie Littlefield, Brain Cancer Advocate
8:45-9:00	BREAK	
9:00-9:20	Keynote - TBA	
9:20-9:30	Australian Research Showcase - Moderated by Barrie Littlefield, Brain Cancer Advocate	
9:30-9:50	Zero Childhood Cancer and PRISM - David Ziegler, Children's Cancer Institute and Claire Wakefield, Sydney Children's Hospital	
9:50-10:10	MNP2 (AIM Brain) Methylation Profiling Collaboration - Nick Gottardo, Princess Margaret Hospital for Children	

10:10-10:30	Development of Novel CAR-T Cells Targeting K27M - Misty Jenkins, The University of Melbourne
10:30-10:50	Clinical CAR-T Cell Therapy for GD2-Positive Malignancies Including Childhood and Adult Gliomas - Michael Brown, South Australia Medical Research Institute
10:50-11:10	Emotion, Science and Hope - Stewart Kellie, Children's Hospital at Westmead; Barrie Littlefield, Brain Cancer Advocate
11:10-11:40	From Bedside to Bench to Bedside: Cancer Researcher Come DIPG Parent - Matt Dun, University of Newcastle
11:40-12:20	LUNCH SERVED IN LOBBY
12:20-12:40	Designing Clinical Trials for Children with DIPG: Tipping the Balance to Pre-Clinical Studies - Kathy Warren, Dana Farber/Boston Children's Hospital
12:40-13:00	DIPG RAPNO - Kenneth Cohen, Johns Hopkins University School of Medicine
13:00-13:30	New Drug Development and ONC201 (Moderated by Stewart Goldman, Lurie Children's Hospital of Chicago) Family and Foundation Networking Session - Steve Healey, The Brooke Healey Foundation; Heidi Feyerherm- Smith, The Cure Starts Now Foundation

	ONC201 Updates - Sharon Gardner, NYU Langone Health	and Love, Unioe Foundation
13:30-13:50	Innovative Trial Design - William Hoos, xCures	
13:50-14:00	BREAK	
14:00-14:20	Biomede - David Castel, Gustave Roussy	
14:20-14:40	Ribocilib Trials Update - Mariko Dewire, Cincinnati Children's Hospital Medical Center	Review of Research Presentations –
14:40-15:00	Genetically Stable Poliovirus Vector Platform for DIPG Immunotherapy - David Ashley, Duke University	Stewart Goldman, Lurie Children's Hospital of Chicago
15:00-15:20	Microdialysis - Sadhana Jackson, National Institutes of Health	
15:20-15:40	DIPG or Not: Diagnosis Definitions and Misdiagnoses – Maryam Fouladi, International DIPG Registry and Cincinnati Children's Hospital Medical Center	

15:40-15:50	BREAK	
15:50-16:00	Closing Session – Gavin Baumgardner, The Cure Starts Now Foundation	
16:30-19:30		Bay Cruise TBA - (reservation only - space limited - open to families and children of guests - requires ticket for all attendees)

Sunday, August 4	Cook Room - Pullman
7:30 - 12:00	CONNECT Consortium Workshop (Members and Invitation Only)
12:00-12:30	LUNCH SERVED IN LOBBY FOR ATTENDEES
12:30-15:30	CONNECT Consortium Workshop (Members and Invitation Only)