











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

www.dipg.org

































































## Speaker's Note

All slide materials must be delivered in final form no later than 6:30 am the day of 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)	Sponsored by Children's Cancer Institute
16:00-17:00		
17:00-20:00	The Cure Starts Now Chapter Dinner (CSN Chapters, Councils and Staff Only)	Hospitality Terrace Open - Terrace, Second Floor Pullman (open to families of attendees) Sponsored by Cure Brain Cancer FOUNDATION Storgminds exceptingsee
20:00-22:00		

Friday, August 2	lbis Room - Pullman	Alternate Location
7:45-8:00 W	Welcome - Ren Pedersen, The Cure Starts Now Australia	
8:00-8:20 W	Recipe for a Cure - Keith Desserich, The Cure Starts Now and the DIPG Collaborative	
8:20-8:40	Review of Current Funded Research Results and Progress (Moderated by Gavin Baumgardner, The Cure Starts Now)	
	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 Intratumoral Drug Penetration and Craniospinal Irradiation – Adam Green. Anchutz Medical Campus
9:40-9:55	Polyamine Pathway Metabolism as a Novel Therapeutic Option for DIPG - Maria Tsoli, Children's Cancer Institute
9:55-10:10	BREAK
10:10-10:25	Targeting DIPG Through Pharmacological Activation of Mitochondrial Biogenesis: an In Vitro and In Vivo Preclinical Study – Cynthia Hawkins, The Hospital of Sick Children
10:25-10:40	MRI Guided Focused Ultrasound: Towards Clinical Translation in DIPG - Saira Alli, The Hospital for Sick Children
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 and Invasion – Maria Vinci, Ospedale Pediatrico Bambino Gesu
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	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

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11:55-12:10		Combinational Strategies Alongside ACVR1 Inhibition in DIPG - Chris Jones, Institute of Cancer Research
12:10-12:25		
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PHOENIX CHILDREN'S		LUNCH AVAILABLE IN LOBBY (Continues into next session)
	WORKING	LUNCH - Cure Fund Grant (Moderated by Gavin Baumgardner)
12:25-13:00 W		of 6 prospective novel research protocols (4 minutes each) with funding of \$50,000 to ers as decided by the Medical Advisory Council.
Sponsored by		L
SNAPGRANT" the uncommon, common app for grant research	12:27	Using Mass Cytometry to Identify Biomarkers in PHGG and DIPG – Maria Vinci, Bambino Gesu Children's Hospital
	12:32	Unlocking the Translational Potential of the DIPG Epigoenome for a Novel Liquid Biopsy – Victor Lu, Mayo Clinic
	12:37	Interaction of Immune System with Oncolytic Measles Virus Therapy of Meddulloblastoma – Sangeet Lal, UCSF Medical Center
	12:42	Development of a New and Effective Pasticity Therapy Against DIPG – Terrance Johns, Telethon Kids Institute
	12:47	Glycomic Tumor Neoantigen Discovery in DIPG – Arun Everest-Dass, Griffith University
	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		A Mouse Model of HIST1H3B/ACVR1 Mutant DIPG - Chris Jones, The Institute of Cancer Research

13:35-13:50	Short-pulse Ultrasound Delivery of Panobinostat for the Treatment of DIPG in Children - Chris Jones, The Institute of Cancer Research	
13:50-14:05	Credentialing an Improved DIPG Mouse Model - Oren Becher, Ann & Robert H. Lurie Children's Hospital	
14:05-14:15	Targeting Neuronal Activity Regulated DIPG Infiltration – Michelle Monje-Deisseroth, Stanford University	
	Review of Previous Cure Fund Grants (Moderated by Gavin Baumgardner)	
14:15-14:30	Cellular Cybercriminals: DIPG Hacks into the Neural Communication Infrastructure – Michelle Monje-Deisseroth, Stanford University	
14:30-14:45	Using Zebrafish to Accelerate DIPG Drug Development – Eric Raabe, Johns Hopkins	
14:45-15:00	Summary – Gavin Baumgardner, Chair Medical Advisory Council, The Cure Starts Now	
15:00-15:15	BREAK	
15:15-15:35 W	Review of DIPG Data Collection Efforts (Moderated by Maraym Fouladi, International DIPG Registry)	
	Cavatica/CBTTC Update - Javad Nazarian, Children's National Medical Center, George Washington University, Universität-Kinderspital; Adam Resnick, Children's Hospital of Philadelphia	
15:35-15:50 W	SIOPe DIPG Registry Update - Dannis van Vuurden, Princess Máxima Center for Pediatric Oncology	
15:50-16:05 W	International DIPG/DMG Registry Update - Maryam Fouladi, Cincinnati Children's Hospital Medical Center	

16:05-16:20 W	CONNECT Collaborative – Maryam Fouladi, Cincinnati Children's Hospital Medical Center	
16:20-16:30	BREAK	
I16·30-17·00	New Institutional Member Forum for International Registry - Maryam Fouladi, Cincinnati Children's Hospital Medical Center	
17:00-17:30		
17:30-21:30		Networking Dinner — The Studio - ticket required (Presentation of Cure Fund Recipients, Collaborative Researcher of the Year and Research Innovator of the Year at 18:30) - short walk with directions
		Sponsored by  THECURESTARTS NOW*

Saturday, August 3	Ibis Room - Pullman	Cook Room - Pullman	Alternate Locati
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	DIPG Registry Collaborations	New Member Forum - Steve Healey, Brooke Healey Foundation	
8:15-8:45		Introduction to the DIPG Symposium - Barrie Littlefield, Brain Cancer Advocate	
8:45-9:00	BREAK		
9:00-9:10	Australian Research Showcase - Moderated by Nick Gottardo, Princess Margaret Hospital for Children		
9:10-9:30	Zero Childhood Cancer and PRISM - David Ziegler, Children's Cancer Institute		
9:30-9:40	The Psychosocial Impacts of Precision Medicine - Claire Wakefield, Sydney Children's Hospital		
9:40-10:00	MNP2 (AIM Brain) Methylation Profiling Collaboration - Nick Gottardo, Princess Margaret Hospital for Children		
10:00-10:20	<b>Development of Novel CAR-T Cells Targeting K27M</b> - Misty Jenkins, The University of Melbourne	e	

10:20-10:40	Clinical CAR-T Cell Therapy for GD2-Positive Malignancies Including Childhood and Adult Gliomas - Michael Brown, Royal Adelaide Hospital	
10:40-11:10	Emotion, Science and Hope - Stewart Kellie, Children's Hospital at Westmead; Barrie Littlefield, Brain Cancer Advocate	
11:10-11:30	From Bedside to Bench to Bedside: Cancer Researcher Come DIPG Parent - Matt Dun, University of Newcastle	
11:30-12:10 Sponsored by THERAPEUTICS	LUNCH SERVED IN LOBBY OUTSIDE IBIS ROOM	LUNCH SERVED IN LOBBY OUTSIDE COOK ROOM
12:10-12:30	Designing Clinical Trials for Children with DIPG: Tipping the Balance to Pre-Clinical Studies - Kathy Warren, Dana Farber/Boston Children's Hospital	
12:30-12:50	DIPG RAPNO - Kenneth Cohen, Johns Hopkins University School of Medicine  Family and Foundation Networkin	
12:50-13:15	New Drug Development and ONC201 (Moderated by Gavin Baumgardner, The Cure Starts Now)	Session - Steve Healey, The Brooke Healey Foundation; Heidi Feyerherm- Smith, The Cure Starts Now and Love, Chloe Foundation
	ONC201 Updates - Sharon Gardner, NYU Langone Health	
13:15-13:35	Innovative Trial Design - William Hoos, xCures	
13:35-13:55	The Changing Regulatory Landscape in Pediatric Clinical Trial Development - Mark Kieran, Bristol-Myers Squibb	
13:55-14:15	Practical Pharmaceutical Development - Wolfgang Oster, Oncoceutics	
14:15-14:30	BREAK	

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14:30-14:50	Biomede - David Castel, Gustave Roussy	CONNECT – Maryam Fouladi, International DIPG Registry and	
14:50-15:10	<b>Ribocilib Trials Update</b> - Mariko Dewire, Cincinnati Children's Hospital Medical Center	Cincinnati Children's Hospital Medical Center	
15:10-15:30	BMI Trials Update - Rachid Drissi, Cincinnati Children's Hospital Medical Center		
15:30-15:50	Genetically Stable Poliovirus Vector Platform for DIPG Immunotherapy - David Ashley, Duke University	Review of Research Presentations – Michelle Monje-Deisseroth, Stanford	
15:50-16:10	Microdialysis - Sadhana Jackson, National Institutes of Health	University	
16:10-16:40	DIPG or Not: Diagnosis Definitions and Misdiagnoses – Maryam Fouladi, International DIPG Registry and Cincinnati Children's Hospital Medical Center		
16:40-16:50	BREAK / EXPANSION SPACING		
16:50-17:00	Closing Session – Gavin Baumgardner, The Cure Starts Now		
17:00-18:00			
18:00-23:15			Bay Cruise TBA - (reservation only - space limited - open to families and children of guests - requires ticket for a attendees)
			Transportation sponsored by  KAZIA



Sunday, August 4	Cook Room - Pullman	
8:15-8:30	CONNECT Consortium Workshop Welcome	
8:30-9:00	CONNECT - Filling the Gap - Maryam Fouladi, CONNECT and Cincinnati Children's Hospital Medical Center	
9:00-9:50	Concept Review, Protocol Development, Database Creation - TBA	
9:50-10:30	International Regulatory and Monitoring - TBA	
10:30-10:45	BREAK	
10:45-12:00	Growing Preclinincal Collaborations	
12:00-12:30	LUNCH SERVED IN LOBBY FOR ATTENDEES	

	Concept C	Overviews - Input and Discussion	
12:30-13:00		MDM2 Inhibition	
13:00-13:20		BMI Medullo	
13:20-13:40		DFMO+AMXT-1501	
13:40-14:00		ACVR1 inhibitor	
14:00-14:15		BREAK	
14:15-15:00	Study Updates (CONNECT1701, CONNECT 1702, CONNECT 1903)		
15:00-15:15	Wrap Up - Growing and Improving		