2019 SNO-SCIDOT
3rd Joint Conference on
Therapeutic Delivery to the CNS

November 20 - 21, 2019
Phoenix, Arizona

CONFERENCE CHAIRS

Michael Vogelbaum, MD, PhD (SCIDOT)
Cleveland Clinic

Nathalie Agar, PhD (SNO)
Dana-Farber Cancer Institute

SCIENTIFIC COMMITTEE

Krystof Bankiewicz, MD, PhD
Ohio State University

Waldemar Debinski, MD, PhD
Wake Forest University

William Elmquist, PhD
University of Minnesota

Raghu Raghavan, PhD
Therataxis, LLC

Nader Sanai, MD
Barrow Neurological Institute

Lisa Shafer, PhD
Cerebral Therapeutics

Mark Souweidane, MD
Weill Cornell Brain and Spine Center

Manfred Westphal, MD
University Hospital Hamburg-Eppendorf
## SNO-SCIDOT 3rd Joint Conference on
**Therapeutic Delivery to the CNS**

**WEDNESDAY, NOVEMBER 20, 2019**

**Co-Chairs:** Nathalie Agar (SNO), Michael Vogelbaum (SCIDOT)

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
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<tbody>
<tr>
<td>6:00 - 8:00am</td>
<td>Registration</td>
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<tr>
<td>7:00 - 8:00am</td>
<td>LIGHT CONTINENTAL BREAKFAST</td>
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| 8:00 - 8:15am | Welcome and Introduction  
  *Michael Vogelbaum, Nathalie Agar*   |

### SESSION 1: BBB PHYSIOLOGY
**Moderator:** Nathalie Agar

<table>
<thead>
<tr>
<th>Time</th>
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| 8:15 - 8:35am    | BBB overview  
  *Lester Drewes*                                                                 |
| 8:35 - 8:55am    | BBB transporters  
  *William Elmquist*                                                                   |
| 8:55 - 9:15am    | CNS pharmacokinetics of chemotherapy  
  *Kathy Warren*                                                                       |
| 9:15 - 9:35am    | Evaluating drug delivery in phase 0 trials  
  *Nader Sanai*                                                                         |
| 9:35 - 9:45am    | Nanotherapeutic targeting of tumor endothelium for enhancing drug delivery past the blood-brain barrier  
  *SCDT-41 G. Praveen Raju*                                                            |
| 9:45 - 9:55am    | *FT-2102 – A potent and selective brain penetrant inhibitor of mutant isocitrate dehydrogenase*  
  *SCDT-42 Maria Ribadeneira*                                                          |
| 9:55 - 10:10am   | BREAK                                                                                   |

### SESSION 2: NEW DRUGS - PRECLINICAL
**Moderator:** Nader Sanai

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
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| 10:10 - 10:30am  | Multivalent agents for GBM therapy  
  *Waldemar Debinski*                                                                  |
| 10:30 - 10:50am  | Local immunomodulation in malignant glioma using motile mesenchymal stem cells  
  *Manfred Westphal*                                                                    |
| 10:50 - 11:10am  | Development of OS2966: a first-in-class, IND approved, anti-CD29 monoclonal antibody for CED  
  *Anne-Marie Carbonell*                                                                 |
| 11:10 - 11:30am  | Novel CAR T cells therapy for GBM  
  *Gianpietro Dotti*                                                                     |
| 11:30 - 11:40am  | MicroRNA nano-cell therapy for glioblastoma  
  *SCDT-46 Muhammad Babar Khan*                                                          |
| 11:40 - 12:00pm  | Evaluating the therapeutic efficacy and convection enhanced delivery (CED) toxicity analysis of CDK4/6 inhibitor palbociclib for diffuse intrinsic pontine glioma (DIPG)  
  *SCDT-48 Dennis Lee*                                                                   |
| 12:00 - 1:00pm   | LUNCH                                                                                   |
WEDNESDAY, NOVEMBER 20, 2019

SESSION 3: CLINICAL TRIALS, PART 1
Moderator: Michael Vogelbaum

1:00 - 1:20pm Oncolytic viral therapy
Frederick Lang

1:20 - 1:40pm Multi-cycle CED for glioma
David Walker

1:40 - 2:00pm CED for DIPG
Mark Souweidane

2:00 - 2:20pm Convection-enhanced delivery of IL13-pseudomonas exotoxin for pediatric diffuse intrinsic pontine glioma
John Heiss

2:20 - 2:30pm A phase I study of convection-enhanced delivery of liposomal-irinotecan (Onivyde) using real-time imaging with gadolinium in patients with recurrent high grade gliomas: results thus far
Karishma Kumar

2:30 - 2:40pm GLITIPNI: A phase 1b clinical trial combining surgical resection with direct intracerebral injection of immune checkpoint inhibitors in patients with recurrent glioblastoma
Johnny Duerinck

2:40 - 2:50pm Overpowering multiple inhibitory immune checkpoints with a single peptide inhibitor enhanced survival in a spontaneous high-grade glioma canine pilot-trial
Michael Olin

2:50 - 3:10pm BREAK

SESSION 4: CLINICAL TRIALS, PART 2
Moderator: Manfred Westphal

3:10 - 3:30pm Update on the Medicenna CED trials
Nicholas Butowski

3:30 - 3:50pm Update on the Tocagen immunogene therapy trials
Michael Vogelbaum

3:50 - 4:10pm Chronic CED of topotecan
Jeffrey Bruce

4:10 - 4:30pm CED of BMP4 in recurrent GBM, results of a phase I trial
Clemens Dirven

4:30 - 4:40pm Ultrasound delivered albumin bound paclitaxel extends survival in malignant glioma models and outperforms ultrasound delivered cremophor paclitaxel in bio-distribution and safety
Adam Sonabend

4:40 - 4:50pm Ultrasound-facilitated drug delivery device for local drug infusion against brain tumors
Ryuta Saito

4:50 - 5:00pm Final data analysis of a pilot study testing the efficacy of using laser interstitial thermal therapy (LITT) to induce temporary disruption of the peritumoral blood brain barrier (BBB) to improve effectiveness of BBB-impermeant chemotherapy in recurrent glioblastoma
David Tran

5:15 - 6:30pm POSTER AND NETWORKING RECEPTION
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<tr>
<td>8:00 - 8:20am</td>
<td>BBB disruption by pulsed electric fields</td>
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<td></td>
<td>Yael Mardor</td>
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<td>8:20 - 8:40am</td>
<td>Ultrasound enabled drug delivery</td>
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<td>Alexandre Carpentier</td>
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<tr>
<td>8:40 - 9:00am</td>
<td>BBB disruption for CNS delivery</td>
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<td>Kullervo Hynynen</td>
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<tr>
<td>9:00 - 9:10am</td>
<td>Low intensity pulsed ultrasound enhances blood-brain barrier opening and improves response to immunotherapy for glioblastoma</td>
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<td>Aria Sabbagh</td>
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<td>9:10 - 9:20am</td>
<td>Brain injury signals systemic immunosuppression through thymic involution</td>
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<td>Katayoun Ayasoufi</td>
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<tr>
<td>9:20 - 9:30am</td>
<td>Towards exclusively local therapy of glioblastoma - engineering IL-12Fc with superior tissue retention and minimal systemic exposure after CNS administration</td>
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<td>Johannes vom Berg</td>
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<td>9:30 - 9:50am</td>
<td>BREAK</td>
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<tr>
<td>9:50 - 10:10am</td>
<td>Unconventional ways to alter interstitial flow rates</td>
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<td>Kip Ludwig</td>
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<td>10:10 - 10:30am</td>
<td>MRI imaging and modeling of connected perivascular spaces</td>
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<td>Malisa Sarntinoranont</td>
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<tr>
<td>10:30 - 10:50am</td>
<td>Biophysical effects of ultrasound as aids to therapeutic delivery</td>
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<td>William Tyler</td>
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<tr>
<td>10:50 - 11:10am</td>
<td>Lessons from simulations and monitoring of intracerebral infusions</td>
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<td>Raghu Raghavan</td>
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<td>11:10 - 11:30am</td>
<td>Role of interstitial fluid flow in drug delivery</td>
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<td>Jennifer Munson</td>
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<td>11:30 - 11:40am</td>
<td>Development of an image-informed mathematical model of convection-enhanced delivery of nanoliposomes for individual patients</td>
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<td>Ryan Woodall</td>
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<td>11:40 - 11:50am</td>
<td>Creating causal capabilities in functional brain mapping using a CED approach</td>
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<td>Walter Block</td>
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<td>11:50 - 1:00pm</td>
<td>LUNCH</td>
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## SESSION 7: NON-TUMOR APPLICATIONS

**Moderator: Raghu Raghavan**

<table>
<thead>
<tr>
<th>Time</th>
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<tbody>
<tr>
<td>1:00 - 1:20pm</td>
<td>Focused ultrasound – opening the blood brain barrier</td>
<td>Ali Rezai</td>
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<tr>
<td>1:20 - 1:40pm</td>
<td>CNS therapeutics and intrathecal delivery</td>
<td>Manuel Sanchez-Felix</td>
</tr>
<tr>
<td>1:40 - 2:00pm</td>
<td>CED for intracerebral hemorrhage and the MISTIE trials</td>
<td>Azam Ahmed</td>
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<tr>
<td>2:00 - 2:20pm</td>
<td>Intraventricular therapy for epilepsy</td>
<td>Eric Distad</td>
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<td>2:20 - 2:30pm</td>
<td>Understanding the effects of molecular size on volume</td>
<td>Julian S. Rechberger</td>
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<td>of distribution in convection-enhanced delivery</td>
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<tr>
<td>2:30 - 2:40pm</td>
<td>K16ApoE enhances Aβ-associated 11C-PiB deposition and PET</td>
<td>Desmond Brown</td>
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<td>signal in APP/PS1 transgenic mice</td>
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<td>2:40 - 3:00pm</td>
<td><strong>BREAK</strong></td>
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## SESSION 8: NEW APPROACHES

**Moderator: Mark Souweidane**

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<thead>
<tr>
<th>Time</th>
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<tbody>
<tr>
<td>3:00 - 3:20pm</td>
<td>A new temozolomide formulation for intracavitary</td>
<td>Manfred Westphal</td>
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<td>chemotherapy</td>
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<td>3:20 - 3:40pm</td>
<td>IPA to IT drug distribution of siRNA</td>
<td>Greg Gerhardt</td>
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<tr>
<td>3:40 - 4:00pm</td>
<td>Novel delivery methods</td>
<td>Jeffry Colella</td>
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<td>4:00 - 4:20pm</td>
<td>Developing treatments for CNS disorders: an industry</td>
<td>Stephen Maricich</td>
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<td>perspective</td>
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<td>4:20 - 4:40pm</td>
<td>Exosome-enabled CNS delivery</td>
<td>Steven Stice</td>
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<tr>
<td>4:40 - 4:50pm</td>
<td>Assessing cerebrospinal fluid flow dynamics in pediatric</td>
<td>Kim Kramer</td>
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<td>patients with central nervous system tumors</td>
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<tr>
<td>4:50 - 5:00pm</td>
<td>In situ delivery of Tumor Treating Fields therapy in</td>
<td>Kristen Carlson</td>
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<td>glioblastoma patients</td>
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<tr>
<td>5:00 - 5:05pm</td>
<td>Closing remarks and adjourn</td>
<td>Michael Vogelbaum, Nathalie Agar</td>
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</tbody>
</table>
SCDT-03 Hyperloaded Poly(2-oxazoline) micelles as personalized drug carriers for brain tumors
Timothy Gershon

SCDT-04 Replacement of microglia by brain-engrafted macrophages prevents memory deficits after therapeutic whole-brain irradiation
Xi Feng

SCDT-05 Developing variable lymphocyte receptors that target pathologically exposed neural ECM to treat glioblastoma
Benjamin Umlauf

SCDT-08 Children’s Brain Tumour Drug Delivery Consortium (CBTDDC)
David Walker

SCDT-14 Enhancing brain retention of a KIF11 inhibitor significantly improves its efficacy in a mouse model of glioblastoma
Steven Rosenfeld

SCDT-15 Disrupting the blood-brain barrier in neuro-oncology: Innovations, achievements, and lessons learned from clinical trials
Alireza Mansouri

SCDT-16 T2-weighted imaging may be indicative of drug distribution in glioblastoma patients
Pamela Jackson

SCDT-18 Nanotherapeutics for neurosurgically-applied drug delivery
Catherine Vasey

SCDT-19 A mechanically-engineered spray to increase brain penetration of chemotherapeutic nanoparticles in the treatment of high-grade gliomas
Phoebe McCrorie

SCDT-20 Adapting engineered cell therapies to understand and overcome glioblastoma resistance using integrated in vivo and ex vivo models
Andrew Satterlee

SCDT-22 Intracerebroventricular delivery of tumor-homing cytotoxic human induced neural stem cells for treatment of brain metastases
Wulin Jiang

SCDT-24 Increasing induced neural stem cell persistence in the tumor resection cavity
Hunter Bomba

SCDT-25 Enhancing the efficacy of drug released from loaded fibrin glue by photochemical internalization (PCI)
Henry Hirschberg

SCDT-26 The role of tumor-derived granulocyte macrophage colony stimulating factor (GM-CSF/CSF2) in regulation of microglia-dependent invasion in gliomas
Kataryzna Poleszak

SCDT-27 TGFβ signaling-induced miRNA participates in autophagic regulation by targeting PRAS40 in mesenchymal subtype of glioblastoma
Chuan Fang

SCDT-28 Targeted nanoparticles for improved drug delivery to medulloblastoma
Joelle Straehla

SCDT-29 Evaluating the feasibility of intranasal FLT delivery for PET imaging of primary brain tumors
Grant Carlisle

SCDT-31 Delayed contrast MRI for high sensitivity depiction of subtle BBB disruption in brain pathologies and as means for monitoring/developing BBB disruption technologies
Yael Mardor

SCDT-32 Neurosurgical delivery of the poly ADP ribose polymerase-1 inhibitor olaparib from a thermo-responsive biodegradable paste potentiates radiotherapy and prolongs survival in high-grade gliomas
Ruman Rahman

SCDT-36 Predictive CED infusion volumes for surgical planning and infusion regime strategies
Clare Killick-Cole

SCDT-45 Magnetic field structure of the Hælo™ pediatric system
Mike Butters

SCDT-47 Effect of uLRFE cognates emulating bioactive substances on animal behavior
Mike Butters

SCDT-50 A rat model for laser interstitial thermal therapy for glioblastoma: effects on peri-ablation blood-brain barrier and implications for post-ablation chemotherapy
Taverekere Nagaraja
The Society for Neuro-Oncology and the Society for CNS Interstitial Delivery of Therapeutics gratefully acknowledge the following sponsors for their support of this program:

**PLATINUM**

- Alcyone LifeSciences, Inc.
- Brainlab
- Infuseon Therapeutics
- MRI Interventions

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