

Project Data Sphere & MCC patient Registry: From Promise to Prototype to Patient

David Miller MD/PhD

Division of Hematology/Oncology and
Department of Dermatology
Massachusetts General Hospital

Michael K. Wong MD/PhD, FRCPC

Department of Melanoma Medical Oncology
MD Anderson Cancer Center

Skin Cancer Outcomes Consortium (SCOUT)

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Disclosures

- David Miller

- I have received honoraria from Pfizer, Merck Sharpe & Dome and Regeneron for contributions to advisory boards

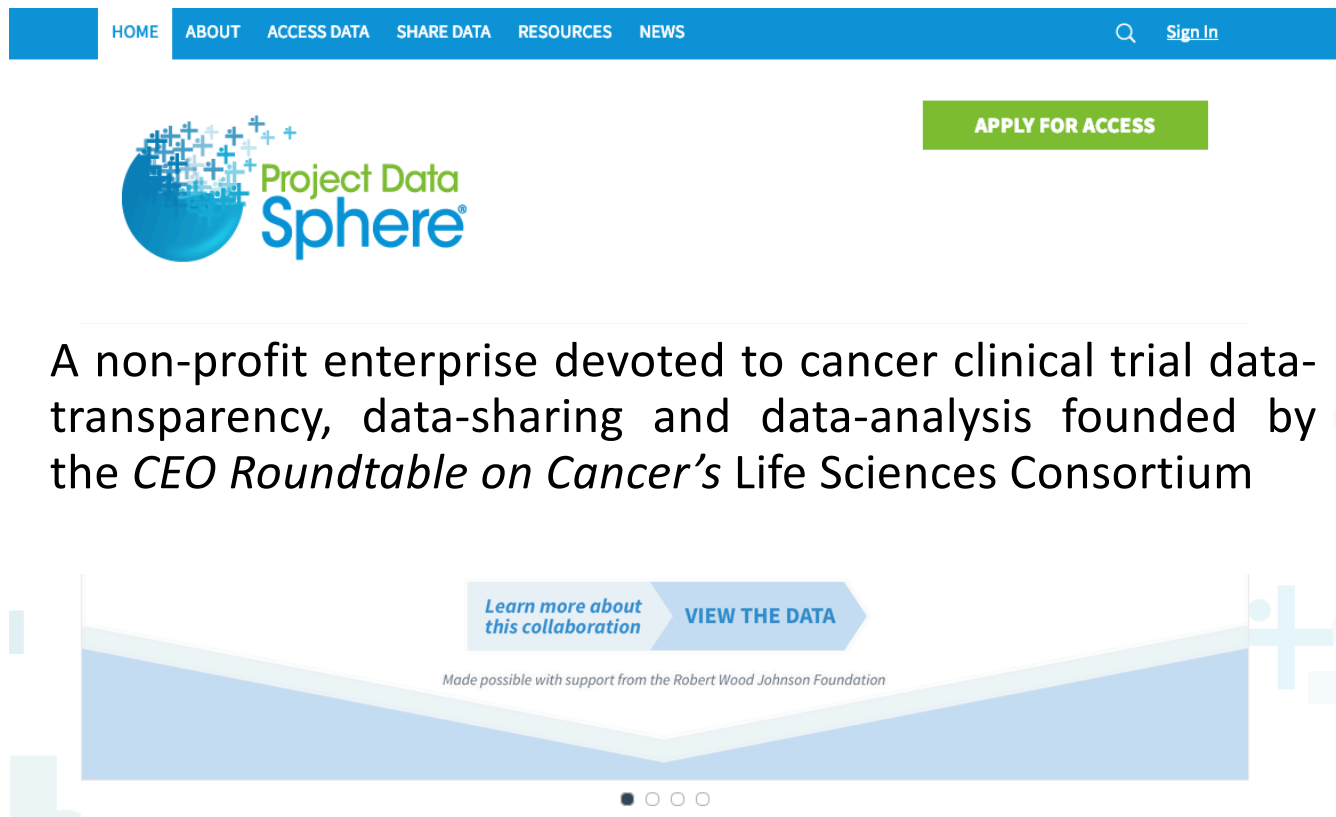
- Michael K Wong

- I have received honoraria from EMD-Serono, Pfizer, Merck, and Regeneron for contributions to advisory boards.
- I receive research support from Merck, Kartos and Bristol Myers Squibb.

Unmet Need

- Clinical outcomes research limited to large administrative data sets or single-institution databases
- Uncertainty in presentation, outcomes and patterns of failure
- Uncertainty in sequencing of available treatments
- Limited approvals for advanced MCC
 - Future approvals for advanced MCC limited by “n”

Origins



Task Force - Members

- **Academia**
- **NIH/NCI**
- **FDA**
- **Sponsor**
- **Project Data Sphere**

Task Force - Members

- **Academia**

- Michael Wong (MDACC) – co-chair
- David Miller (MGH) – co-chair
- Paul Nghiem (UW)
- Ken Tsai (Moffit)
- Chris Bichakjian (Mich)
- Kelly Harms (Mich)
- Kristina LaChance (UW)
- Lauren Haydu (MDACC)
- Vishal Patel (GW)
- Arthur Sober (MGH)

- **NIH/NCI**

- Isaac Brownell

- **FDA**

- Meredith Chuck

- **Sponsor**

- Hao Zhang (EMD Serono)
- Zhen Su (EMD Serono)
- Catherine Symonds (EMD Serono)

- **Project Data Sphere**

- Bill Louv
- Martin Murphy
- Megan Granda

Task Force – Current & Near-Term Objectives

- Identify the objectives and applications of an MCC Patient Registry
- Select and Develop a Data Collection Instrument
- Develop and Execute an Implementation Strategy

Task Force - Objectives



Merkel Cell Carcinoma Registry

David M. Miller MD PhD, Michael K. Wong MD PhD
October 8, 2018

Executive Summary

The Merkel Cell Carcinoma (MCC) Patient Registry is a national multi-institutional collaborative effort that will prospectively follow and record outcomes and events in MCC patients. MCC is the prototypical rare tumor, and this Registry will trail blaze new methodologies that will enable multiple investigators to examine real world outcome data in real time. Deliverables from the Registry include: (i) precise patient stratification into risk categories, (ii) identification of best practices, (iii) revelations about optimal sequence and combinations therapies, (iv) uncovering low incidence toxicities and (v) the generation of novel testable hypotheses. Importantly, the Registry offers a way forward in the yet-unsolved dilemma of drug development for rare tumors since the Registry's design will allow for the creation of highly defined patient-level data that can be used as a robust comparator for single arm Phase I-II clinical trials.

The MCC Task Force comprises members from academic medical centers, the drug industry, the NIH and FDA. Project Data Sphere, LLC provides a secure, open-access data sharing platform and comprehensive support to optimize research performance and ensure rigorous and timely results. The Registry is currently in the planning stage but will be based on a REDCap database integrated into the host institution's electronic medical record. We plan to have the first patient accessioned on Project Data Sphere's data platform in Q1 of 2019.

Objectives And Applications

- **Natural History Study**

- Precisely characterize the presentation and natural history of Merkel Cell Carcinoma
- Identify best practices in MCC
- Identify accurate covariates that are prognostic in MCC

- **Patient Level Data As Real World Evidence for Drug Development**

- Function as external data set
- Tool for safety assessment
- RWE to support sBLA/sNDA

Data Capture Tool – REDCap



- REDCap is a secure web application for building and managing databases
- It can be used to collect virtually any type of data, including
 - HIPAA-compliant environments
 - 21 CFR Part 11
- The REDCap consortium is a vast support network of collaborators

Implementation Strategy

- Task 1: Build Consensus Data Fields
- Task 2: Rollout and Scalability Plan

Task 1: Consensus Data Fields

Data Collection Instruments		
	Instrument name	Fields
	Protected Health Information	10
	Non PHI Clinical Characteristics Of The Patient (PMH, Social Hx, etc)	60
	Clinical Characteristics of the Initial Presentation - Features of the primary lesion, I	22
	Imaging - Initial Staging	11
	Surgical Therapy - Initial Excision	10
	Lymph Node Evaluation - Initial Work Up	26
	Metastases - Initial Staging	11
	Pathology - Initial Staging	67
	Staging - Initial	55
	Radiation Therapy	18
	Medical Anti-Neoplastic Therapy	29
	Surgical Therapy - Additional (i.e. after initial staging)	8
	Adverse Events	18
	Subject Status	13
	Clinical Benefit/Response	12

Data Collection Instruments		
	Instrument name	Fields
	Recurrence	19
	Progressive Disease	17
	Lab Results - MCC Polyoma Virus Serum Oncoprotein Antibody Test	5
	Lab Results - Hematological	14
	Lab Results - Electrolytes/Renal	12
	Lab Results - Cardiac	6
	Lab Results - Endocrine	7
	Lab Results - LDH	4
	Imaging - Restaging/Ongoing Follow up	12
	Pathology - Followup	36
	Palliative Care/Hospice	3
	Death Details	17
	Clinical And Translational Research - Protocol Documentation	3
	Translational Research - Biospecimen Collection Documentation	8
	Calculated Fields For Preliminary Statistics	15

Task 2: Rollout and Scalability Plan

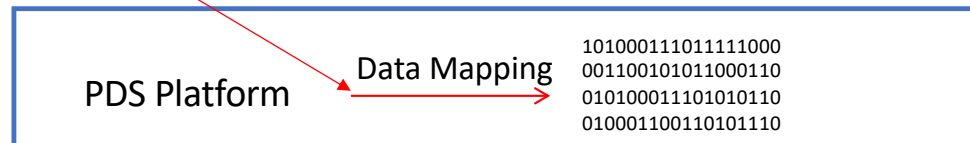
Task 2: Rollout and Scalability Plan

Registry of Merkel Cell Carcinoma Patients

Tiered Model for Data Access

- I. Closed Access
- II. Limited Access
- III. Open Access

Data Access Groups
George Washington
MD Anderson
MGH



Next Steps

- Test Beta Version
- Develop Data Quality Plan
- Develop Data Usage Agreements

Support

- MCC Community
- Project Data Sphere
 - Research Administration
 - Data Management
 - PDS vast network

Is there a role for a PDS supported high-risk cSCC Registry?

Acknowledgements

- Task Force Members
- PDS
- FDA
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- MMIG