Project Data Sphere and Ongoing Multi-Institutional Database Efforts

David M. Miller MD/PhD

Division of Hematology/Oncology and Department of Dermatology

Massachusetts General Hospital

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Disclosures

- David Miller
  - I have received honoraria from Pfizer, Merck Sharpe & Dome, Sanofi and Regeneron

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
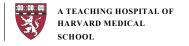 - Future approvals for advanced MCC limited by "n"

PDS MCC Tumor Registry

A non-profit enterprise devoted to cancer clinical trial data-transparency, data-sharing and data-analysis founded by the *CEO Roundtable on Cancer's* Life Sciences Consortium

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)
  - Manisha Thakuria (BWH/DFCI)
- **NIH/NCI**
  - Isaac Brownell

- **Sponsor**
  - Hao Zhang (EMD Serono)
  - Zhen Su (EMD Serono)
  - Catherine Symonds (EMD Serono)
- **Project Data Sphere**
  - Bill Louv
  - Martin Murphy
  - Ravikumar Komandur

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

Rare Diseases: Natural History Studies for Drug Development
Guidance for Industry

DRAFT GUIDANCE

Pilot Study

- Clarify
  - What data elements to collect
  - How to code the data
  - How to standardize the information collection in a way to facilitate analysis

Data Collection Instruments and Fields

Instrument Name	Fields
Patient Characteristics	71
Presentation And Initial Staging	83
Subject Status	10
Lesion Information	237
Medical Antineoplastic Therapy	50
Adverse Events	16
Lab Results	60
Imaging	12
Research Protocol Documentation	3
Biospecimen Collection Documentation	12

Pilot Study – Part 1, MGH

Data collected after patient signs consent

Prospective Capture

100111010111010100101011

N = 34

N = 258

100111010111010010101101011101001010101

Retrospective Capture
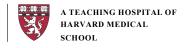
Data collected under a waiver of consent

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Pilot Study – Part 2, MGH, MD Anderson, GW

Registry of Merkel Cell Carcinoma Patients

| 🏠 Project Home | ☰ Project Setup | 👤 User Rights | 👥 Data Access Groups |

Data Access Groups	Number of records in group
MGH	292
MD Anderson	0
George Washington	0

MCC Registry - Data Collection & Access Plan

Registry of Merkel Cell Carcinoma Patients

| 🏠 Project Home | ≣ Project Setup | 👤 User Rights | 👥 Data Access Groups |

Data Access Groups	Number of records in group
MGH	292
MD Anderson	0
George Washington	0
DFCI	0
Michigan	0
Moffitt	0
Univ of Washington	0

Limited PHI

De-identification →

Tiered Model for Data Access
I.   Closed Access

PDS Platform → Data Mapping →
```
1010001110111111000
0011001010110001110
0101000111010101110
010001100110101110
```
De-identified Data

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Next Steps

- Part 2 of the Pilot Study

- Develop Data Quality Plan

- Develop Data Usage Agreements

Support

- MCC Community

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

Acknowledgements

- Task Force Members

- PDS

- FDA

- Global Oncology Big Data Alliance (GOBDA)