



CURE-OS FACTOR 2025 Breakout

A Blueprint for Translating Single-Cell Discoveries into Better Osteosarcoma Treatments

MIB FACTOR Osteosarcoma Conference, June 2025, Salt Lake City, Utah

EXECUTIVE SUMMARY

Executive Summary

The CURE-OS osteosarcoma single-cell working group held a breakout session at the 2025 MIB FACTOR conference aimed at accelerating the translation of single-cell discoveries into clinically meaningful advances for osteosarcoma. With over 45 participants spanning basic science, computational biology, translational research, clinical oncology, and patient advocacy, the session brought together researchers, clinicians, and patients/families for collaborative problem-solving across three key areas: (1) **research and clinical data collection**; (2) **leveraging existing data**; and (3) **experimental model evaluation**.

The breakout was anchored by two central goals:

1. **To build a shared framework** for translating single-cell discoveries into clinically meaningful progress in osteosarcoma.
 - Identify critical obstacles to translating single-cell technologies in osteosarcoma
 - Define the essential data, models, and infrastructure our community needs
2. **To foster cross-disciplinary collaboration** that connects single-cell insights with actionable research strategies and clinical translation.
 - Lay the groundwork for future initiatives (e.g., grants, trials, manuscripts) that we can pursue together over the next year

Participants identified critical barriers, formulated priorities, and co-developed strategies toward practical solutions that will advance these goals. Across roundtables, participants emphasized collaboration, standardization, and data-model integration as essential drivers of meaningful progress. This summary highlights key takeaways and proposes early deliverables to mobilize shared efforts across the osteosarcoma community in support of the priorities identified in this breakout.

Roundtable Highlights & Key Takeaways

1. Prioritization of Research and Clinical Data Collection (Leads: Anand Patel, Emanuela Palmerini)

Focus: What data are most urgently needed to answer high-impact biological and clinical questions in osteosarcoma, and how do we design ideal datasets to support these goals?

Discussion Highlights:

- Continued focus on addressing the following barriers:
 - Inconsistent clinical annotation practices across institutions
 - Logistical and infrastructure limitations for prospective biospecimen collection
 - Lack of standardized data-sharing pipelines for clinical data
- Data supporting prospectively validated biomarkers for risk stratification is key
 - Integrate existing biomarkers into clinical trials to prospectively validate and compare
 - Incorporate clinical, genomic, imaging, and pathology data within new AI approaches



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- Standardize sample collection protocols (biospecimen and clinical data) across institutions for longitudinal and multi-omic profiling
 - Identify minimum essential clinical annotations for biospecimens to support research (e.g., treatment regimens, outcomes, imaging, etc.)
 - Prioritize the collection of cases that reflect diverse clinical scenarios and rare subtypes

Key Priorities Identified:

- Advance biologically-driven risk stratification
- Develop systems to address siloed cohorts
- Identify gold standard dataset for harmonized clinical and research data
- Focus on temporal (serial, longitudinal) data

2. Leveraging Existing Data (Lead: Corey Weistuch)

Focus: How do we increase transparency, accessibility, and collaborative use of existing osteosarcoma single-cell datasets?

Discussion Highlights:

- Developing a centralized repository of osteosarcoma single-cell data would be beneficial
 - Centralized annotations and visualizations; integration with GEO
- Inventory of existing datasets and biospecimens across institutions (in process)
- Address technical, legal, and ethical barriers to data sharing (clinical, biospecimen, molecular), including consents, platform interoperability, etc.

Key Priorities Identified:

- Table of known datasets across institutions (in process)
- Vision and governance plan for a centralized CURE-OS single-cell data platform

3. Experimental Model Evaluation (Lead: Dan Regan)

Focus: What are the best preclinical models to test single-cell derived hypotheses, and how can we credential and refine them for clinical translation?

Discussion Highlights:

- Expert panel committed to longitudinal study of the predictive validity of models
 - Match models to translational use cases (e.g., immunotherapy, resistance, metastatic disease)
 - Sharing negative studies
 - Establish benchmarks for model fidelity and consensus characterization across model species
 - Setting preclinical bar for translation
- Human/model integration - iterative cycle as we leverage novel technologies to better characterize human tumors
 - Address limitations in model predictiveness with rigorous cross-validation and profiling
- Leverage existing databases (e.g., [Cancermodels.org](https://cancermodels.org))



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Key Priorities Identified:

- Framework for matching model systems with translational use cases
 - Preclinical data matrix (shared repository including performance across drug classes)
 - Credentialing criteria for models
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Consensus Priorities & Action Items

Based on these discussions and community input, the following vision and initiatives emerged as near-term opportunities to accelerate progress:

Vision: Establish a platform to support the use of multi-omic data to characterize osteosarcoma patient tumors, identify prognostic and therapeutic biomarkers, and align them with relevant experimental models toward clinical translation.

Primary goals and immediate deliverables:

1. Aggregate and centralize existing single-cell data
 - a. Deliverable: Single-cell data aggregation and hosting plan (see Tab 2)
 2. Define a prioritized, gold standard dataset for prospective data collection
 - a. Deliverable: Ranked wishlist for prospective data collection (see Tab 3)
 3. Framework for matching experimental models to therapeutic translation
 - a. Deliverable: Model matching matrix (see Tab 4)
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Call to Action

We invite all participants to remain engaged and provide insight into the immediate deliverables.