



Rare Cancers Prevention and Interception Virtual Workshop May 7–8, 2024

Agenda

DAY 1 (10:00 AM to 5:30 PM)

10:00-10:15 AM **Welcome**
Altaf Mohammed, Ph.D., Workshop Co-Organizer, Division of Cancer Prevention, NCI

Introductory Remarks
Kimryn Rathmell, M.D., Ph.D., Director, NCI
Phil Castle, Ph.D., Director, Division of Cancer Prevention, NCI

10:15-11:00 AM **Plenary Talk**
Brigitte C. Widemann, M.D., Center for Cancer Research, NCI
Overview of rare cancers and highlights of NCI efforts

SESSION I • Overview of rare cancers: current status

Co-Chairs: Taylor Sundby, M.D., and Robert Shoemaker, Ph.D.

11:00-11:30 AM **Sharon Savage, M.D.**, Division of Cancer Epidemiology and Genetics, NCI
The NCI Cancer Prevention and Interception Clinic: conception and vision

11:30 AM-12:00 PM **Uri Tabori, M.D.**, The Hospital for Sick Children, University of Toronto
Clinical and biological updates on replication repair deficiency syndromes

12:00-12:30 PM **LUNCH**

12:30-1:00 PM **Payal Khincha, M.B.B.S., M.S.H.S.**, Division of Cancer Epidemiology and Genetics, NCI
Multidisciplinary approaches to germline cancer predisposition: the Li-Fraumeni syndrome perspective

1:00-1:30 PM **Paul P. Liu, M.D., Ph.D.**, National Human Genome Research Institute
Inherited predisposition to hematologic malignancies — NIH natural history study of RUNX1-FPDMM

1:30-2:00 PM **Samra Turajlic, M.D., Ph.D.**, The Francis Crick Institute
Replaying the tape of malignant transformation in VHL disease

2:00-2:30 PM **Panel Discussion**

DAY 1 (10:00 AM to 5:30 PM)**2:30-2:45 PM BREAK****Session II • Emerging opportunities for rare cancers prevention and interception***Co-Chairs: Altaf Mohammed, Ph.D., and Payal Khincha, M.B.B.S., M.S.H.S.*

2:45-3:15 PM Sarah Blagden, M.D., Ph.D., University of Oxford, London
Metformin pharmacoprevention trial in Li-Fraumeni syndrome (MILI)

3:15-3:45 PM Yurong Song, Ph.D., Frederick National Laboratory for Cancer Research
Preclinical studies for Lynch syndrome cancer prevention and interception

3:45-4:15 PM Irene Ghobrial, M.D., Dana Farber Cancer Institute
Early detection and interception in myeloma

4:15-4:45 PM David Largaespada, Ph.D., University of Minnesota
Proteomic and immunopeptidomic characterization of the MPNST cell surface for targeted therapy discovery

4:45-5:30 PM Panel Discussion

5:30 PM Adjourn
Kajal Biswas, Ph.D., Workshop Co-Organizer, Division of Cancer Prevention, NCI

DAY 2 (9:45 AM to 6:00 PM)

9:45-10:00 AM **Welcome**
Sagar Ghosh, Ph.D., MBA, Workshop Co-Organizer, CDMRP, DoD

Introductory Remarks
Colonel Sarah B. Goldman, Ph.D., Director, CDMRP, DoD

SESSION III • Networking — scientists, advocacy groups, and healthcare providers

Moderators: Sagar Ghosh, Ph.D., MBA, and Payal Khincha, M.B.B.S., M.S.H.S.

10:00-10:20 AM **Patient Advocate Corrie Painter, Ph.D.**, Broad Institute
 Driving discoveries in rare cancers through social connections

10:20-10:40 AM **Patient Advocate Isis Sroka, Ph.D.**, Franconi Anemia Research Fund
 Empowering Fanconi anemia advocates to drive innovation in rare cancer research

10:40-11:00 AM **Patient Advocate Katrin Ericson, Ph.D.**, RUNX1 Research Program
 Synergizing science and advocacy: strategies to optimize collaboration and improve patient engagement
Additional discussion to follow in Session VI

Session IV • Rare cancers clinical trials updates

Co-Chairs: Martha Donoghue, M.D., and Anju Singh, B.V.Sc, Ph.D.

11:00-11:30 AM **Theodore W. Laetsch, M.D.**, Children's Hospital of Philadelphia
 Rare tumor clinical trials in the Children's Oncology Group

11:30 AM-12:00 PM **Alice Chen, M.D.**, Division of Cancer Treatment and Diagnosis, NCI
 Rare tumor trials in a phase I clinic: ideal match

12:00-12:30 PM **LUNCH**

12:30-1:00 PM **Eduardo Vilar-Sanchez, M.D., Ph.D.**, MD Anderson Cancer Center
 The current landscape of cancer interception clinical trials in Lynch syndrome

1:00-1:30 PM **Andrea M. Gross, M.D.**, Center for Cancer Research, NCI
 Challenges of clinical trial design for prevention in rare diseases

1:30-2:00 PM **Martha Donoghue, M.D.**, The Food and Drug Administration
 Regulatory considerations for clinical trials in rare cancers

DAY 2 (9:45 AM to 6:00 PM)**SESSION V • Challenges and opportunities in rare cancers research: biology, targets, and prevention***Co-Chairs: Kajal Biswas, Ph.D., and Grace Ault, Ph.D.*

2:00-2:30 PM	Kevin B. Jones, M.D. , University of Utah Interrogating epigenomic mechanisms in rare cancers with mouse genetic models
2:30-3:00 PM	William Foulkes, Ph.D., M.B.B.S. , McGill University Rare and unexplained familial tumors: past, present, future
3:00-3:15 PM	BREAK
3:15-3:45 PM	Steven Rhodes, M.D., Ph.D. , Indiana University Decoding NF1 peripheral nerve sheath tumor heterogeneity: insights from genetically engineered mice
3:45-4:15 PM	Thomas D. Wang, M.D. Ph.D. , University of Michigan Early detection of Barrett's neoplasia using multi-valent interactions
4:15-4:45 PM	Robert Shoemaker, Ph.D. , Division of Cancer Prevention, NCI Feasibility of using frameshift mutations in peripheral blood as a biomarker for surveillance of Lynch syndrome

SESSION VI • Brainstorming and future directions*Moderators: Anirban Das, M.B.B.S., M.D., D.M., and Altaf Mohammed, Ph.D.*

4:45-6:00 PM	Panelists: Brigitte Widemann, Robert Shoemaker, Sharon Savage, Martha Donoghue, Anirban Das, Payal Khincha, David Largaespada, William Foulkes, Theodore Laetsch, Katrin Ericson, Alice Chen, Paul Liu, Andrea Gross
6:00 PM	Closing Remarks and Adjourn Kajal Biswas, Altaf Mohammed, Sagar Ghosh