



# INSTITUTE FOR RNA MEDICINE

## newsletter

Spring 2016

## iRM Gains HMS Initiative Status

The Institute for RNA Medicine will soon expand to become a Harvard Medical School Initiative. With the approval in early May from the HMS Initiatives, Programs, Centers and Institutes Standing Committee, the iRM will work with RNA researchers across the HMS Quad and affiliated hospitals.

“We’re extremely happy to report that the iRM will be able to leverage the experience and expertise of the broader Harvard community,” said iRM Director Frank Slack, PhD. “As the iRM continues to grow and as we continue to discover important new roles for non-coding RNA, it will be beneficial to capitalize on the tremendous opportunities for scientific collaboration that exist across the Harvard medical system.”

## RNAMECINE2016 Symposium



RNAMECINE2016, the second annual symposium of the BIDMC Cancer Center’s Institute for RNA Medicine, brought together 12 of the field’s most prominent scientists on April 27 for a day of illuminating presentations and provocative discussion.

The event once again attracted an overflow audience and provided a program of engaging talks from a stellar scientific lineup of the best minds in RNA medicine. Throughout the day, discussions focused on how this once-mysterious genetic material cuts across all aspects of biomedical science and all disease areas, notably cancer, but also metabolic diseases, neurological conditions and some of public health’s most pressing issues. “We’ve found that the list of non-coding RNAs is vastly greater than what we ever discovered 20 years ago, when the first regulatory roles were described for this mysterious genetic material,” said Slack, in the day’s opening remarks. “The sequencing of the human genome opened our eyes to a new universe of regulatory molecules that are numbering in the tens of thousands – and we don’t know where that number is going to end.”

Speakers included iRM founding members Pier Paolo Pandolfi, MD, PhD, Director of the BIDMC Cancer Center; and Jeffrey Saffitz, MD, PhD, BIDMC Chief of Pathology. Scientific presentations were given by Anders M. Näär, PhD; Xandra O. Breakefield, PhD; Anna Marie Pyle, PhD; David P. Bartel, PhD; Sangeeta N. Bhatia, MD, PhD; Lin He, PhD; Howard Y. Chang, MD, PhD; George Q. Daley, MD, PhD; Joshua Mendell, MD, PhD; Amy Pasquinelli, PhD; Anastasia Khvorova, PhD; and iRM Director Frank Slack, PhD.

*from the desk of*

### Frank J. Slack, PhD

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### Recent News

[Weird Loops of Genetic  
Material Help Cause Cancer](#)

From *Forbes*, March 31, 2016

Dr. Pier Paolo Pandolfi’s work on circular RNAs could uncover causes and treatments for blood cancers

[The Stars of the Cell](#)

Dr. Frank Slack, iRM Director, on the emerging field of non-coding RNA and its potential impact on medicine and health

[Current and Future Trends in  
RNA Medicine](#)

The iRM and BIDMC’s Tech Ventures Office co-sponsored a panel discussion in November 2015

# Long Non-coding RNA Mouse Models Created by iRM Members

iRM members Pier Paolo Pandolfi, MD, PhD, and John Rinn, PhD, are setting out to generate gold standard mouse models for long non-coding RNA (lncRNA) genes. Their prior work has identified many candidate lncRNAs and pseudogenes that represent a new class of cancer associated genes they term "Onco-lncRNAs" and "Tumor suppressor lncRNAs." Armed with nearly a decade of research on these mysterious molecules they have honed in on two key candidates: one onco-lncRNA and one tumor suppressor lncRNA. These candidates have been found in large cancer datasets, including The Cancer Genome Atlas (TCGA), to be up- or down-regulated across many cancer types.

Going all in they have generated both loss- and gain-of-function mouse models for these new non-coding RNA cancer genes. Moreover, they have generated compound models to see how these lncRNAs impact classic cancer pathways and influence tumor development. Specifically, they are focusing on KRAS which is mutated in a high percentage of colorectal, pancreatic and lung cancers, and PTEN, a critical tumor suppressor frequently disrupted in multiple tumor types. Collectively, the development of such an important resource will allow us to test the necessity and sufficiency of lncRNAs in cancer, an endeavor not yet taken in vivo at this scale.

Importantly, these new lncRNA genes are being studied in the context of over 35 years of knowledge of the KRAS and PTEN pathways. Dr. Pandolfi's experience in these models and the new lncRNAs identified by the Rinn lab are set to identify insights with the potential to achieve significant breakthroughs into the roles of lncRNAs in cancer.

## RNA Technology Showcase Events

The iRM was pleased to welcome speakers from four companies offering RNA-related technologies and services to our offices in March 2016. On March 1, Lihan Zhou, PhD, from MiRXES spoke on "Discovering Circulating MicroRNA Biomarkers Using a Highly Controlled qPCR Work flow" and Mark Behlke, MD, PhD, of Integrated DNA Technologies presented on "Improved CRISPR gene editing using chemically-modified Alt-R RNA oligonucleotides." On March 8, Peter Mouritzen, PhD, of Exiqon, Inc. spoke about "Applications of Locked Nucleic Acids: From detection to functional analysis of RNAs" and David Grotzky, PhD, of Abcam presented on "Multiplex miRNA Profiling Using Firefly Technology." The next RNA Technology Showcase will be held on Tuesday, June 7 in room CLS 421 at 3 Blackfan Circle in Boston. It will feature presentations by Kit Fuhrman, PhD, of NanoString, and Alex Forrest-Hay, PhD, and Noah Alberts-Grill, PhD, of Affymetrix.


## iRM Director Receives Award

On May 5, 2016, Dr. Frank Slack received the MicroRNA Innovator Award at the Precision Medicine Symposium USA 2016 from GeneExpression Systems, Inc. and the Appasani Research Conferences & Educational Institute.

## Sponsorship Opportunities

The Institute for RNA Medicine welcomes inquiries from individuals and companies interested in supporting RNA medicine. Donations to the iRM can be directed toward the overall operating costs for a particular event, including our annual Symposium. Please contact us for more information.

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April 28, 2016  
Thanks again to our speakers and attendees for a very successful symposium yesterday #rnamedicine2016. Please join us again next year.

April 25, 2016  
At the iRM we are actively exploring the non-coding genome [www.bidmc.org/irm](http://www.bidmc.org/irm) #DNADay16

April 21, 2016  
From the Slack Lab: miR-34a Silences c-SRC to Attenuate Tumor Growth in Triple Negative Breast Cancer [bit.ly/1qFrdcP](http://bit.ly/1qFrdcP)

## Events

### iRM Seminar Series

D. Ashley Hill, PhD, Children's National Medical Center  
May 24, 2016, 3 PM  
CLS 421, 3 Blackfan Circle

### RNA Technology Showcase

Kit Fuhrman, PhD, NanoString  
Alex Forrest-Hay, PhD, and Noah Alberts-Grill, PhD, Affymetrix  
June 7, 2016, 3 PM  
CLS 421, 3 Blackfan Circle

### RNAMEDICINE2017

April 27, 2017  
Joseph B. Martin Conference Center, Harvard Medical School  
Registration opens Jan. 2017

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