

FALL/WINTER 2011

# DISCOVERY

## LRI LAUNCHES GROUNDBREAKING RESEARCH INITIATIVE AT 11TH ANNUAL SCIENTIFIC CONFERENCE

Forum for Discovery 2011 Highlights Significant Scientific Progress



With so many of the nation's top scientific minds in one room, the intellectual energy ran high as cutting edge results were shared and ideas exchanged. Discoveries, advances and works in progress generated spirited interchange and excitement in the collective achievements. But when Margaret Dowd, LRI President and CEO

took the podium to announce a bold new international research initiative, the mood was electric. The Distinguished Innovators Initiative aims to uncover

the fundamental root causes of lupus, begin the drive to a cure, and open the door to prevention.

**The initiative was launched with an \$8 million grant from Bloomberg Philanthropies.**

Michael R. Bloomberg, philanthropist and Mayor of New York City gave the following quote for the press release announcing the program nationwide:

**“Lupus is a too-often overlooked disease that affects 1.5 million Americans in this country, and thousands of New Yorkers. This grant will fuel the work of the Distinguished Innovators Initiative and further LRI’s demonstrated track record in significantly advancing medical discovery. I am confident this investment will help advance efforts to find both the basic causes of this complex disease and the cure.”**

### In This Issue....

PAGE 2 Executive Corner

PAGE 3 Distinguished Innovators Applauded

PAGE 3 Great Minds Don't Always Think Alike

PAGE 4 Effective Advocacy Gets Results

PAGE 5 Industry Forum

PAGE 6 Highlights of Forum Presentations

PAGE 7 Two Days to Think Just About Lupus!

PAGE 8 Planned Giving

PAGE 8 LupusTrials.org: #1 Clinical Trials Resource

Dear Lupus Research Institute Friends,

As we close Year One of a new decade at the LRI, we have many extraordinary achievements we are proud to share with you.

This year's Annual Forum for Discovery was truly about collaboration — providing a forum for America's most talented scientists to learn from each other, sharing their findings and their ideas to spark their thinking throughout the coming year.

It is their pioneering work that allowed us to make an announcement that may well transform the future of lupus research — **closing in on the causes and going for the cure! An extremely generous \$8 million grant from Bloomberg Philanthropies** is making it possible for the LRI to launch a trailblazing new research program. Our Distinguished Innovators Initiative will award \$1 million grants to top researchers who can bring the world's most creative and novel scientific approaches to uncover the fundamental root causes of lupus. While existing treatments and compounds in the pipeline aim to suppress the effects of lupus, this global initiative seeks to stop lupus in its tracks, reverse the disease process, and achieve prolonged drug-free remission — a cure.



We thank keynote dinner speaker Dr. Marc Tessier-Lavigne, president of Rockefeller University and former Chief Scientific Officer of Genentech, for his inspiring optimism. The story on Page 3 may well convince you of his thesis — drug development can be seen as a glass “half-full” of promise for major advances in lupus.

Don't miss Page 4 to learn what our National Coalition of lupus patient advocacy groups throughout the country has accomplished in making lupus a national priority. Stretching coast to coast and virtually every urban center in between, the Coalition effected real change on the Hill that is raising awareness of lupus among physicians as well as patients and reducing disparities in care among the underserved. We also recognize the tremendous work of our supporters who raise funds throughout the year to allow LRI to deliver what may be the best service we can provide patients in the long-run — the innovative science that finds the answers.

We at the LRI share Dr. Tessier-Lavigne's excitement in anticipating the future. We are embarking on a new era of exploration pointing toward finding the cure for lupus. The initial backing from Bloomberg Philanthropies gives us a running start. With the continued help of our dedicated supporters, we can go all the way — to achieving “Life Without Lupus.”

A handwritten signature in black ink that reads "Margaret P. Dond".

## Distinguished Innovators Applauded by LRI Board, Scientific Advisors and Patients



“Prudent risk is needed to move towards even deeper insights, to find new treatments. Whether we will succeed we cannot know, but if we don’t try and try our very best, we will never get there.”

*Dr. William Paul, LRI Scientific Advisory Board Chairman, Member of National Academy of Sciences*

“After a decade of LRI accomplishment, we asked ourselves, where would we like to be a decade from now? We envisioned Distinguished Innovators as a high-risk but high-reward program attracting the best scientists to be free to develop the most novel concepts.”

*Dr. Peter Lipsky, LRI Scientific Advisory Board Member, Editor-in-chief of Nature Reviews Rheumatology (right)*



“It is because of all our researchers’ hard work that we earned the Bloomberg Philanthropies’ confidence. Looking out at on all your brilliant minds, speaking as a patient and on behalf of all families affected by lupus, I thank you from the bottom of my heart.”

*Jennie DeScherer, Lupus Patient/Advocate & Board Member of LRI and S.L.E.Lupus Foundation (above)*

## Great Minds Don’t Always Think Alike



The Forum for Discovery brought together America’s scientific up-and-comers with fresh ideas, seasoned researchers with years of experience, industry pros and clinicians on the ground to look at lupus drug development from all angles.

### Keynote Speaker Sees Drug Development as “Half-Full”

Keynote speaker Marc Tessier-Lavigne brought two sides of the drug development equation together as former Chief Scientific Officer of biotechnology giant Genentech and current president of Rockefeller University, a world-renowned center for clinical and basic research.

Dr. Tessier-Lavigne acknowledged why industry’s current situation can be seen as a glass half-empty. Yet he maintains, “It’s time to look at pharmaceutical drug development as half-full. As waves of scientific understanding break, so too will waves of drug development.”

He attributes his guarded optimism largely to the growing collaboration among industry, academia, clinicians and the private sector in translating research into clinical medicine. “Private organizations like the Lupus Research Institute have helped academia to identify drug targets, and industry is rapidly building on that work to do what they do best — develop new drugs and make them very well.”

**“We are in a golden age of drug discovery.”**

*Marc Tessier-Lavigne*



Through years of advocacy at the federal level, the LRI's National Coalition of state and local lupus groups has worked successfully to secure the funding needed to develop the "Eliminating Health Disparities in Lupus Initiative," a collaboration among the federal offices of Minority Health, Women's Health, and the Surgeon General. The Initiative aims to reduce barriers to care through programs educating physicians and other healthcare professionals

about lupus, its diagnosis and its treatment. Because of this educational program, the general practitioner will now have lupus on the list of possible diagnoses to consider.

This work is a testament to what the private sector can accomplish in partnership with government. To further advance this critical program, the U.S. Senate Appropriations Committee recently voted to allocate an additional \$1 million to the Lupus Initiative. When this final bill passes, total funds we worked hard to secure will reach \$4.6 million.



"Having walked the halls of Congress relentlessly, Coalition members are deeply gratified at the continued support of the federal government in reducing health disparities in lupus," noted LRI President Margaret Dowd. "Like many patient advocates, ours bring the power of unbridled passion for the lupus cause. But what made this effort so successful is the ability to harness that passion with an understanding of how to work effectively with government. Together we made sure that lupus was on the Congressional radar, where it has continued to command appropriation support despite today's difficult financial environment."

More advocacy work remains to be accomplished as issues threatening treatment access continue to arise. Currently, the LRI and its Coalition are leveraging its power in numbers to petition the United Kingdom's National Institute for Health and Clinical Excellence (NICE) to reverse their recommendation not to cover the cost of Benlysta®. Additionally, on behalf of beneficiaries reliant on Medicare Part D for essential medications, we are urging the Congressional Joint Select Committee on Deficit Reduction to protect the Part D program as federal budget cuts are considered.

## And on the State Level

In their own communities Coalition members throughout the country are continually finding ways to increase awareness, support, and access to quality medical care for people with lupus. For example, LRI Coalition members — the Lupus Agencies of New York State — are fighting for Medicaid Patient Protections, Lupus Programs and Taxpayer Gifts, and legislation opposing limitations to coverage for approved and investigational treatments.



"Under the Coalition's leadership, patient advocacy organizations throughout New York have united to support cost-efficient initiatives that preserve medical professionals' rights to make treatment decisions that are in the best interest of their patients," said Kathleen Arntsen, LRI Coalition member and lupus patient.

## Industry Forum – “Challenges in Translational Research in Lupus”

Another highlight of the conference, the Industry Forum, drew on key representatives of the lupus community to tackle “Challenges in Translational Research in Lupus.” The panel was moderated by Peter Lipsky, MD, a world immunology leader with a background in academia and government, as well as experience with the private sector as an LRI scientific advisor.

Speakers shared outlooks on issues in translational medicine that must be addressed to accelerate lupus drug development.



### Academia

Back in academia after forming a biotechnology company, Dr. Matthew Holers (University of Colorado) emphasized interaction between academia and industry. If Dr. Holers had his way, academic scientists would spend a sabbatical in industry to see every step from bench to bedside.



Dr. Matthew Liang (Harvard Medical School) attributes a “bottleneck” in approving biomarkers to the difficulty in recruiting a large enough sampling of lupus patients. He called for timely sharing of patient records and clinical trial data as a key solution.

### Pharma and Biotech Industry

Bringing a regulatory mindset to Genentech from his years at the U.S. Food and Drug Administration, Dr. Jeff Siegel sees biomarkers as key to identifying patients, providing early evidence of drug benefits and dosage, and to evaluating treatment.



Dr. Henry Hess (EMD Serono) emphasized the need to test a scientific hypothesis earlier in humans rather than to project solely from success in animals.

### Clinical Practice

Dr. Daniel J. Wallace (Cedars-Sinai Medical Center) asked industry to design trials that: ensure participation from academic centers that reach underserved patients; include patients with mild to moderate disease; and reflect standard of care.



## Highlights of Forum Presentations: Building on a Growing Knowledge Base

### What Can Biomarkers Tell Us?

Biomarkers, potential indicators to monitor disease progress and treatment success in clinical trials, are among the most challenging and critical areas of lupus. To date, LRI has supported 20 researchers aiming to discover new biomarkers for the many different manifestations of lupus.

Three LRI investigators have developed novel non-invasive approaches to diagnosing lupus kidney disease that may provide safer, more accurate alternatives to a surgical biopsy.



**Dr. Joshua Thurman** (University of Colorado) and his colleagues developed a novel diagnostic tool known as an investigational contrast agent that when used with magnetic resonance imaging (MRI), can detect proteins in the kidney that indicate lupus disease in mice. Using a biomarker to monitor disease

may substitute for surgical biopsy. “In addition to being potentially safer, the MRI approach should be more sensitive than biopsy by looking at the entire kidney rather than just a small sample. Based on these encouraging results, we plan to investigate the agent in lupus patients.”



**Dr. Chandra Mohan** (UT Southwestern Medical Center) and his LRI grant collaborator Dr. Chaim Putterman (Albert Einstein College of Medicine) have discovered that the levels of certain proteins in the urine of lupus mice can be used to monitor kidney disease. With LRI funding, Drs. Mohan and

Putterman are conducting tests in humans, using the urine of people with lupus to determine the value of these proteins as biomarkers or indicators of disease severity. Predicting flares, when patients’ symptoms suddenly worsen, and monitoring their response to treatment, can help better manage their disease.

### LRI Among Experts at FDA Conference on Biomarkers

*Two Investigators Present Discoveries Funded by Lupus Research Institute*

In September, the LRI attended an invitation-only U.S. Food and Drug



Administration workshop focusing on the development of biomarkers to predict acute worsening of disease, called a flare, and their potential for accelerating lupus clinical trials and new drug approvals.

Discussion highlighted LRI-funded discoveries by Drs. Emily Baechler Gillespie at University of Minnesota’s Institute of Human Genetics, and Maureen McMahon at UCLA David Geffen School of Medicine. Based on an LRI-funded longitudinal study Dr. Gillespie’s team developed a biomarker to predict disease flare that has been licensed for commercial development. Work presented by Dr. McMahon showed promise in using biomarkers to predict risk for disease flare and worsening of atherosclerosis for lupus patients.

The Lupus Research Institute was one of the earliest supporters of lupus biomarker research, launching a strategic research program in novel biomarker discovery nearly a decade ago. Since then, the LRI has invested \$5.6 million in 20 biomarker studies, many of which are now concluding with promising results.

Two Days to Think Just About Lupus!



Best Talent... Best Ideas... Best Results...



THE ENERGY IN SYNERGY



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New York, NY 10001

[www.LupusResearchInstitute.org](http://www.LupusResearchInstitute.org)

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Please **keep us in the loop** and alert us if your email or mailing address has changed. Just send an email to [lupus@lupusny.org](mailto:lupus@lupusny.org)!

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## Planned Giving: Where There's a Will, There's a Way

Planned and deferred gifts to the S.L.E. Lupus Foundation and Lupus Research Institute can help to continue a legacy of support and service to benefit forthcoming generations.

Tax advantages and income benefits for donors are also possible. Planned gifts typically come from a donor's assets rather than income, and can be either outright or deferred.

Types of gifts include:

- **Bequests:** including the S.L.E. Lupus Foundation in your Will
- **Charitable Trusts:** either remainder or lead, which may be funded with highly appreciated securities
- **Special Gifts:** appreciated securities; insurance policies; retirement plan assets; real estate holdings; and other real property

For more information, please contact Andrea O'Neill at 212.685.4118 or email [aoneill@lupusny.org](mailto:aoneill@lupusny.org).

## LupusTrials.org: #1 Website for Clinical Trial Information

Sponsored by the LRI, LupusTrials.org is the number one site to learn about clinical trials. The site earned its status with educational materials, an interactive search function that makes it easier to find the right trial, and the how-to's of enrollment.



As #1, we must be doing something right! But we'd like your input on how we can continue to best meet patients' needs. Please visit [LupusTrials.org](http://LupusTrials.org) to fill out a brief questionnaire. Thanks for your input!



**Thanks Go to Federal Employee Supporters of the CFC**

Whose Hard-Earned Dollars Are Working Harder to Fuel Lupus Discovery