

**2013 – Year of Italian Culture in the U.S.**

## **Symposium on**



## **STATE OF THE ART FOR A GLOBAL ALLIANCE**

within the framework of the Intergovernmental agreement between the Department of Health and Human Services of the United States of America and the Ministry of Health of Italy and with the collaboration of NIH, USA and ISS, Italy

**December 16th 2013**

**8.00am - 5.15pm**

**Embassy of Italy - Auditorium**  
**3000 Whitehaven St., NW -**  
**Washington, DC 20008**



*Speakers from US and Italian central administrations, leading research Institutes, Academia, private no-profit and community based associations, and from the industry will address state of the art research, current and future diagnostic and treatment developments, values and implications of the alliance between professionals and patients and their families promoted in the two Countries. Decision makers will illustrate where rare diseases stand in the health policy agenda and how the forthcoming European research framework program will facilitate transatlantic joint research and innovation.*

*Domenica Taruscio, Director of the National Centre for Rare Diseases, Istituto Superiore di Sanità and Stephen C. Groft, Director of the Office of Rare Diseases Research, National Center for Advancing Translational Sciences, National Institutes of Health will lead the dialogue.*

To register, [CLICK HERE](#)



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2013 ANNO DELLA CULTURA ITALIANA  
YEAR OF ITALIAN CULTURE

The event is sponsored by



8.00 – 8.30	Registration		
8.30 – 9.30	<p>Welcome address and opening remarks</p>	<ul style="list-style-type: none"> <li>• Claudio Bisogniero</li> <li>• Fabrizio Oleari (televideoconference)</li> <li>• Stephen C. Groft</li> <li>• Domenica Taruscio</li> </ul>	<p>Ambassador of Italy to the US</p> <p>President, Istituto Superiore di Sanita', ISS</p> <p>Office of rare diseases research, National Center for Advancing Translational Sciences, NIH</p> <p>National Center for Rare Diseases, Istituto Superiore di Sanita', ISS</p>
<p>9.30 – 11.30 Rare Diseases research: organization and opportunities in a global perspective</p>	<p>NIH Undiagnosed Diseases Program/Network (NHGRI)</p> <p>Public-private Partnership in support of rare diseases research: achievements and challenges</p> <p>Rare Diseases Clinical Research Network</p> <p>Rare Diseases Registries as tools for clinical research</p> <p>The Drug Repurposing Program</p> <p>Therapeutics for Rare and Neglected Diseases Program and Bridging Interventional Development Gaps Program</p> <p>Mitochondrial role in muscle dystrophy (the case of Ullrich's disease and of Bethlem myopathy)</p>	<ul style="list-style-type: none"> <li>• William A. Gahl</li> <li>• Lucia Monaco</li> <li>• Rashmi Gopal-Srivastava</li> <li>• Erica Daina</li> <li>• Christine Colvis</li> <li>• John McKew</li> <li>• Alessia Angelin</li> </ul>	<p>Clinical Director, NHGRI Director, NIH Undiagnosed Diseases Program</p> <p>Chief Scientific Officer, Fondazione Telethon</p> <p>Office of Rare Diseases Research (ORDR), NCATS/NIH</p> <p>IRCCS Istituto di Ricerche Farmacologiche "Mario Negri" Clinical Research Center for Rare Diseases</p> <p>Therapeutics Discovery Program, NCATS/NIH</p> <p>Acting Scientific Director, NCATS/NIH</p> <p>Center for Mitochondrial and Epigenomic Medicine, Children's Hospital of Philadelphia, University of Pennsylvania</p>
Q&A and Discussion	<b>11.30 – 11.45 Coffee break</b>		

<p>11.45 – 12.15 The Transatlantic perspective</p> <p>12.15 - 13.30 Research advances in support of service delivery</p> <p>Q&amp;A and Discussion</p>	<p>The Role of the EU in the International Rare Diseases Consortium (IRDiC) and opportunities for contributions in Horizon 2020</p> <p>The Down Syndrome Patient Registry and Mitochondrial Disease Research Initiatives</p> <p>Non pharmaceutical treatment of rare brain tumors</p> <p>Next generation sequencing and <i>zebrafish</i> in neuromuscular diseases</p> <p>Tissue on a Chip Program, Extracellular RNA</p>	<ul style="list-style-type: none"> <li>• Errol G. Levy</li>   <li>• Danuta Krotoski</li>   <li>• Michelle Alonso-Basanta</li>   <li>• Chiara Manzini</li>   <li>• Danilo Tagle</li> </ul>	<p>First Secretary, deputy Head, Science, Technology and Education, Delegation of the EU to the US</p> <p>Intellectual and Developmental Disabilities Branch, Eunice Kennedy Shriver, NICHD/NIH</p> <p>Diversity Search Advisor, Dept. Radiation Oncology, University of Pennsylvania</p> <p>Assistant Professor, Dept. of Pharmacology and Physiology, and Integrative Systems Biology, The George Washington University</p> <p>Associate Director for Special Initiatives, NCATS</p>
<b>13.30 – 14.30</b>	<b>Lunch offered by Zambon Pharmaceuticals</b>		
<p>14.30 – 16.15 Therapeutic solutions, pharmaceutical research and challenges 1: general and regulatory issues</p>	<p>Orphan Products Designation, Medical Devices, and Grants Program</p> <p>R&amp;D for rare diseases: challenges and opportunities</p> <p>Patient Registries and Biospecimen Repositories</p> <p>Small and medium enterprises' opportunities and challenges for rare disease/orphan drugs development in the US</p>	<ul style="list-style-type: none"> <li>• Gayatri Rao</li>   <li>• Marco Sardina</li>   <li>• Yaffa Rubinstein and Matthew McAuliffe</li>   <li>• Gianfanco Fornasini</li> </ul>	<p>Director, Office of Orphan Products Development, FDA</p> <p>R&amp;D director, Zambon Pharmaceuticals</p> <p>Office of Rare Diseases Research (ORDR), NCATS/NIH</p> <p>Senior Vice President of Scientific Affairs of Sigma-Tau Pharmaceutical</p>

Q&A and Discussion	The interregional network of rare diseases of Northwest Italy: opportunities for novel strategies of public health, assistance, and research	<ul style="list-style-type: none"> <li>• Dario Roccatello, Simone Baldovino</li> </ul>	Regional center coordinator, Piedmont Region
16.15 – 17.00 Pharmaceutical research and challenges 2: specific issues  Q&A and Discussion	<p>NIAID's resources for vaccine and drug development</p> <p>Exon Specific U1RNA for the treatment of Spinal Muscular Atrophy</p> <p>Public and private players: a thriving business model for patients and investors: why pharmaceutical companies are entering in this space?</p>	<ul style="list-style-type: none"> <li>• Cristina Cassetti</li> <li>• Franco Pagani</li> <li>• Paolo Barbanti</li> </ul>	<p>Program Officer Acute Viral Diseases Program, Virology Branch, Division of Microbiology and Infectious Diseases, NIAID/NIH</p> <p>Human Molecular Genetics, Group Leader, International Centre for Genetic Engineering and Biotechnology, Trieste</p> <p>CEO of Pharma &amp; Biotech Advisors srl</p>
17.00 – 17.15	Conclusions and the way forward	<ul style="list-style-type: none"> <li>• Chris Austin (TBC)</li> <li>• Ranieri Guerra</li> </ul>	Director, NCATS/NIH Scientific Attache', Embassy of Italy