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## **58<sup>th</sup> ASH ANNUAL MEETING**

December 3-6, 2016  
San Diego, CA

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Session Type Oral Presentation  
Session Title 112. Thalassemia and Globin Gene Regulation: Molecular Mechanisms of Thalassemia

**Date Monday, December 5, 2016**  
**Time 5:15 p.m. PT**  
Location Room 7AB (San Diego Convention Center)

**Title 1012 - Potential Therapeutic Applications of Jak2 Inhibitors and Hif2a-ASO for the Treatment of  $\beta$ -Thalassemia Intermedia and Major**  
*Carla Casu*<sup>1\*</sup>, *Paraskevi Rea Oikonomidou, M.D.*<sup>2\*</sup>, *Vania Lo Presti*<sup>1\*</sup>, *Mariam Aghajan, PhD*<sup>3\*</sup>, *Shuling Guo, PhD*<sup>3\*</sup>, *Abdulmalik Osheiza*<sup>1\*</sup>, *Luca Melchiori*<sup>4\*</sup>, *Pedro Ramos*<sup>5\*</sup> and *Stefano Rivella, PhD*<sup>2</sup>

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<sup>4</sup>Adaptimmune Ltd, Abingdon, United Kingdom  
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Session Type Oral Presentation  
Session Title 112. Thalassemia and Globin Gene Regulation: Molecular Mechanisms of Thalassemia

**Date Monday, December 5, 2016**  
**Time 5:30 p.m. PT**  
Location Room 7AB (San Diego Convention Center)

**Title 1013 - Developing a Galnac-Conjugated TMPRSS6 Antisense Therapy for the Treatment of  $\beta$ -Thalassemia**  
*Mariam Aghajan, PhD*<sup>1\*</sup>, *Carla Casu, PhD*<sup>2\*</sup>, *Vania Lo Presti*<sup>2\*</sup>, *Sheri Booten*<sup>1\*</sup>, *Brett P. Monia, PhD*<sup>1</sup>, *Stefano Rivella, PhD*<sup>2</sup> and *Shuling Guo, PhD*<sup>1\*</sup>

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Session Type	Oral Presentation
Session Title	112. Thalassemia and Globin Gene Regulation: Molecular Mechanisms of Thalassemia
Date	<b>Monday, December 5, 2016</b>
Time	<b>5:45 p.m. PT</b>
Location	Room 7AB (San Diego Convention Center)
Title	<p><b>1014 - Adult Hemoglobin Production, Chain Rebalance, and Splice Correction in IVS2-745 Beta-Thalassemia Patient Cells Using 2'-O-Methoxyethyl Splice-Switching Oligos</b></p> <p>Alisa Cheung Dong<sup>1,2*</sup>, Valentina Ghiaccio<sup>1*</sup>, Irene Motta, MD<sup>3*</sup>, Shuling Guo, PhD<sup>4*</sup>, Raechel Peralta, MS<sup>4*</sup>, Coralea Stephanou, PhD<sup>5*</sup>, Paola Delbini, PhD<sup>6*</sup>, Connie Chen<sup>7*</sup>, Soteroula Christou<sup>8*</sup>, Marina Kleanthous, PhD<sup>9*</sup>, Maria Domenica Cappellini, MD<sup>10</sup>, Osheiza Abdulmalik, DVM, MS<sup>11</sup>, <b>Laura Breda, PhD<sup>12*</sup></b> and Stefano Rivella, PhD<sup>12</sup></p> <p><sup>1</sup>Hematology, Children's Hospital of Philadelphia, Philadelphia, PA  <sup>2</sup>Graduate School of Medical Sciences, Weill Cornell Medical College of Cornell University, New York, NY  <sup>3</sup>Rare Diseases Center, Department of Medicine and Medical Specialities, Ca' Granda IRCCS Fondazione Ospedale Maggiore Policlinico, Milan, Italy  <sup>4</sup>Ionis Pharmaceuticals, Inc., Carlsbad, CA  <sup>5</sup>Molecular Genetics of Thalassemia Department, The Cyprus Institute of Neurology and Genetics, Nicosia, Cyprus  <sup>6</sup>Rare Diseases Center, Department of Medicine and Medical Specialities, Ca' Granda IRCCS Fondazione Ospedale Maggiore Policlinico, Milan, Italy  <sup>7</sup>Weill Cornell Medical College, New York  <sup>8</sup>Thalassaemia Center, Archbishop Makarios III Hospital, Nicosia, Cyprus  <sup>9</sup>The Cyprus Institute of Neurology and Genetics, Nicosia, Cyprus  <sup>10</sup>Department of Medicine and Medical Specialities, Department of Clinical Science and Community Health, Rare Diseases Center, Fondazione IRCCS "Ca' Granda", Ospedale Maggiore Policlinico, University of Milan, Milan, Italy  <sup>11</sup>Division of Hematology, The Children's Hospital of Philadelphia, Philadelphia, PA  <sup>12</sup>Children's Hospital of Philadelphia, Philadelphia, PA  <sup>1</sup>Ionis Pharmaceuticals, Inc., Carlsbad, CA  <sup>2</sup>Children's Hospital of Philadelphia, Philadelphia, PA</p>

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