Fifth Eastern European Conference for Rare Diseases and Orphan Drugs  
“Rare Diseases in the Focus of Personalized Medicine”

First All-Russian Conference for Rare Diseases and Rarely Used Medical Technologies “Lifeline”

2-4 July 2010

Preliminary program

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<tr>
<th>Time</th>
<th>Hall 1</th>
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<tr>
<td>8.00-10.00</td>
<td>Registration</td>
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<tr>
<td>10.00-12.00</td>
<td><strong>Oppenning ceremony. Greetings.</strong></td>
<td><strong>Presentation from the Russian Ministry of Health and Social Development of the Russian Federation (Nataliya N. Tochilova) – theme is specified</strong></td>
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<td><strong>Presentation from the Federal service on supervision in sphere of public health services and social development (lecturer is specified) - Problems of rare diseases treatment and orphan drugs registration in the Russian Federation</strong></td>
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<td>12.00-12.45</td>
<td>Press conference</td>
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<td>12.00-13.00</td>
<td>Dinner</td>
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<td>13.00-15.30</td>
<td>Plenary session №1. <strong>Rare Diseases in the Focus of Personalized Medicine</strong> Potential speakers:</td>
<td>14.00-18.00 2nd Tyrosinemia type 1 symposium <strong>Chairmen:</strong> Prof. Petr V. Novikov (Russia) Dr. Grant Mitchell (Canada) Dr. Patrick McKiernan (UK)</td>
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</table>

- **Antoni Montserrat** (SANCO – Health and Consumer Protection, Public Health and Risk Assessment, Health Information, European Commission, Brussels, Belgium)
- **Catherine Berens** (Scientific Officer, Medical and Public Health Research Unit, European Commission) - ?
- **Kerstin Westermark** (Chair of the Committee for Orphan Medicinal Products of the European Medicines Agency – EMEA, Sweden)
- **Steve Groft** (Director, Office of Rare Diseases at National Institutes of Health, USA) - ?
- **Tim Cote** (Director at FDA, Office of Orphan Products Development, USA) - ?
- **Emmanuel Chantelot** (European Biopharmaceutical Enterprises, EFPIA)
  - Russia (lecturer is specified)

15.30-16.00 Coffee break. Poster session №1
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| 16.00-18.30| Plenary session №2. **Best Practice in Field of Rare Diseases and Personalized Medicine**  
Potential speakers:  
- **Yann Le Cam** (CEO European Organisation for Rare Diseases – EURORDIS, EU) - ?  
- **Christel Nourissier** (President of Prader-Willi (France) and Secretary General of EURORDIS, France) - ?  
- **Segolene Ayme** (Director of research at the French Institute of Health and Medical Research (INSERM), Executive Manager of Orphanet, Leader of the Rare Diseases Task Force established by the European Commission, DG Public Health and Consumers’ protection, France)  
- **Domenica Taruscio** (Director of the Italian National Center Rare Diseases and responsible of the Italian National Registry Rare Diseases, Italy)  
- **Sonja van Weely** (Scientific officer of the Dutch Steering Committee Orphan Drugs, Netherlands)  
- **Edmund Jessop** (Medical Adviser for the National Specialist Commissioning Advisory Group, UK) | 1    |
| 20.00-23.00| Gala-Dinner                                                            |      |

**II day – Plenary session**

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<td>13.30-15.00</td>
<td>Dinner</td>
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| 15.00-17.00| Plenary session №3. **Rare Diseases – Future, Perspectives, Challenges of Health Care**  
- **Policy** (Prof. Rumen Stefanov, Director of Information Centre for Rare Diseases and Orphan Drugs of Bulgarian Association for Promotion of Education and Science)  
- **Prevention** (Dr. Gerard Loeber, Head of Lab for Infectious Diseases and Perinatal Screening at National Institute for Public Health and Environment, Netherlands)  
- **Diagnostics** (Prof. Anna Tylki-Szymanska, Department of Metabolic Diseases of the Children’s Memorial Health Institute, Poland) - ?  
- **Alexander A. Apazov** (President of Russian Pharmaceutical Association, Russia) - *Possibilities of medicinal provisions of patients with rare diseases in Russia*  
- **Treatment** (lecturer is specified)  
- **Ilkka Kantola** (Dr. Department of medicine Central Hospital University of Turku, Finland) - ?  
- **Rehabilitation** (lecturer is specified) | 1    |
<p>| 17.00-17.30| Coffee break. Poster session №2                                       |      |</p>
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<tr>
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| 17.30-19.30 | **Plenary session №4. Modern capabilities in diagnostics and treatment of rare diseases**  
Prof. Alexander G. Rumyantsev (Russia)  
Prof. Vladislav S. Baranov (Russia)  
Prof. Alexander V. Polyakov (Russia)  
Dr. Ekaterina Yu. Zakharova (Russia) |
| 20.30    | Walk and supper by the ship                                                     |
## II day – Sectional sessiones

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| 9.00-10.30 | 2<sup>nd</sup> Tyrosinemia type 1 symposium

**Chairmen:**
- Prof. Petr V. Novikov (Russia)
- Dr. Grant Mitchell (Canada)
- Dr. Patrick McKiernan (UK)

Lysosomal storage diseases – Gaucher disease
*(program is specified)*

Storage diseases – Wilson’s disease, hemochromatosis, porphyrias

**Chairman:**
- Prof. Alexey A. Sokolov (Russia)

| 10.30-11.00 | Coffee break. Poster session №3                                        | Lysosomal storage diseases – Fabry disease, MPS
*(program is specified)*

Organic acidemias and acidurias
*(program is specified)*

| 11.00-12.30 | 2<sup>nd</sup> Tyrosinemia type 1 symposium

**Chairmen:**
- Prof. Petr V. Novikov (Russia)
- Dr. Grant Mitchell (Canada)
- Dr. Patrick McKiernan (UK)

Lysosomal storage diseases – Niemann-Pick «C» disease
*(program is specified)*

Phenylketonuria and galactosemia
*(program is specified)*

**Chairwoman:** Dr. Lidia V. Lyazina, Dr. Natalia V. Zuravskaya (Russia)

| 12.30-13.00 | Coffee break. Poster session №4                                        | Rare diseases of the blood – hemophilia
*(program is specified)*

Rare respiratory system diseases – pulmonary arterial hypertension

**Chairwoman:** Prof. Irina E. Chazova (Russia)

| 13.00-14.30 | Rare diseases of the blood – thalassemic syndromes

**Chairman:**
- Prof. Kudrat M. Abulkadyrov (Russia)

Mucoviscidosis
*(program is specified)*

Rare diseases and conditions in nephrology

**Chairman:**
- Dr. Sergey G. Borovoy (Russia)

| 10.30-11.00 | Coffee break. Poster session №3                                        | Rare respiratory system diseases – pulmonary arterial hypertension

**Chairwoman:** Prof. Irina E. Chazova (Russia)

| 11.00-12.30 | Rare diseases of the blood – thalassemic syndromes

**Chairman:**
- Prof. Kudrat M. Abulkadyrov (Russia)

Mucoviscidosis
*(program is specified)*

Rare diseases and conditions in nephrology

**Chairman:**
- Dr. Sergey G. Borovoy (Russia)

| 12.30-13.00 | Coffee break. Poster session №4                                        | Rare diseases of the blood – chronic myeloid leukemia

**Chairman:**
- Prof. Alexander G. Rumyantsev (Russia)

Primary immunodeficiency

**Chairwoman:** Prof. Natalia M. Kalinina (Russia)

| 13.00-14.30 | Rare diseases of the blood – chronic myeloid leukemia

**Chairman:**
- Prof. Alexander G. Rumyantsev (Russia)

Primary immunodeficiency

**Chairwoman:** Prof. Natalia M. Kalinina (Russia)

| 14.30-15.30 | Rare circulatory system diseases - ?                                   | Rare circulatory system diseases - ?

**Chairman:**
- Prof. Alexander G. Rumyantsev (Russia)

| 15.30-16.30 | Rare circulatory system diseases - ?                                   | Rare circulatory system diseases - ?

**Chairman:**
- Prof. Alexander G. Rumyantsev (Russia)
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| 9.00-10.30 | Rare endocrine diseases - hyperparathyreosis  
*Chairman:* Dr. Ilya V. Slepcov (Russia) | Rare nervous system diseases - myasthenic syndromes  
*Chairman:* Prof. Vadim G. Pishchik (Russia) | Rare infections - ?                  |
| 10.30-11.00| Coffee break. Poster session №3             |                                            |                                  |
| 11.00-12.30| Rare endocrine diseases - ?                 | Rare nervous system diseases – multiple sclerosis  
*(program is specified)*                |                                  |
| 12.30-13.00| Coffee break. Poster session №4             |                                            |                                  |
| 13.00-14.30| Rare tumors - ?                            | Rare digestive system diseases - Crohn’s disease  
*Chairwomen:* Prof. Elena. A. Kornienko  
Dr. Natalia K. Zaletova,  
Dr. Olga V.Fedorovih,  
(Russia)  
*(program is specified)* |                                  |

**Ill day**

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| 9.00-12.00 | Sectional session review  
Results of poster session  
Certificates kommitment  
Conference closing |                                  |
| 12.00-12.30| Coffee break |                                  |
2nd Tyrosinemia type 1 symposium

Chairmen:

Professor Petr V. Novikov, Department of Clinical Genetics, Institute of Pediatrics and Child surgery, Moscow, Russia
Dr Grant Mitchell, Service de Génétique Médicale, Hôpital Ste-Justine, Montreal, Canada
Dr Patrick McKiernan, consultant paediatrician, Liver Unit, Children’s Hospital NHS Trust, Birmingham, United Kingdom

Speakers:

Dr Svetlana I Polyakova, Senior Researcher Research Center of Children’s Health Department of Gastroenterology and hepatology Russian Academy of Medical Sciences Moscow, Russia
Associate Professor Elisabeth Holme, Department of Clinical Chemistry Sahlgrenska University Hospital, Gothenburg, Sweden
Dr Carlos Dionosi-Vici, Division of metabolism, Bambino Gesu Hospital, Rome, Italy
Dr Guillern Pintos-Morell, Departments of Pediatrics; University Hospital, Badalona, Spain
Professor Philippe Robaey, University of Ottawa, Children’s hospital of Eastern Ontario, Ottawa, Canada
Dr Francjan van Spronsen, Department of Pediatrics, University Hospital of Groningen, The Netherlands

Preliminary Program
Sectional session “Pulmonary arterial hypertension”

Chairmen: Prof. Irina E. Chazova (Moscow)

1. Film about Pulmonary arterial hypertension – 10 min.
2. Aetiology and pathogenesis of pulmonary arterial hypertension.
   S.N. Nakonechnikov (Moscow) – 25 min.
3. Diagnostics of pulmonary arterial hypertension.
   T.V. Martynyuk (Moscow) – 25 min.
4. Treatment of pulmonary arterial hypertension.
   Prof. Irina E. Chazova (Moscow) – 30 min.
Фильм по болезни Ниманна-Пика типа С – 10 мин.
Подходы к диагностике и лечению больных с болезнью Ниманна-Пика типа С. Международный опыт. Проф. Фредерик Седель (Frederic Sedel) (Франция) - 45 мин.