Russian Federation

Prof. Alexey Sokolov
Russian Federation
(not less than 1 million person should have rare diseases)

Square – 17 075 400 Sq.km
Regions – 83

Population – 141 867 540
Nationalities – 180

Hospitals – about 10 000
Physicians – about 707 000
Basic positions of the Russian legislation on the Public Health

• Act 41.1 of Constitution of Russian Federation
  “Every person has a right to health protection and medical care”…regardless of frequency of occurrence of his disease.

• Act 17 of Fundamental principles of legislation of Russian Federation about health protection of subjects (Federal law from August, 22 2004, № 122-ФЗ)
  “State provides health protection of subjects independent from their sex, race, nationality, language, class origin, post, place of residence, religious commitment, creed, membership in public associations and other conditions. State guarantees protect of subjects from any form of discrimination is due to their diseases.”
How it looks now in Russia?

• Medical care is focused on management of socially relevant disease, but individual person with rare disease is face to face with his disease.

• Patient with rare disease:
  – may buy required drug,
  – may take it for free if he is related to benefit category of patients having a right to social benefits:
    • *patients having a right to additional pharmacological support in according to Federal law №122-Ф3 from 22.08.2004 – «federal benefit persons»;
    • *patients having a right to consegcional pharmacological support in according to regional legislative acts – «regional benefit persons»;
  – may suffer from diseases included in *state program of pharmacological support of patients with 7 rare and expensive diseases* that had been realized from 2008.
State program of pharmacological support of patients with 7 rare and expensive diseases (2008)

<table>
<thead>
<tr>
<th>Disease</th>
<th>Drugs</th>
</tr>
</thead>
<tbody>
<tr>
<td>Hemophilia</td>
<td>Octanate®, Haemoctin STD®, Recombinate®, NovoSeven®, Oktanaif F®, Immunate®, Immunine®, Kogenate FS®</td>
</tr>
<tr>
<td>Mucoviscidosis</td>
<td>Pulmozim®</td>
</tr>
<tr>
<td>Pituitary nanism</td>
<td>Norditropin®</td>
</tr>
<tr>
<td>Gaucher disease</td>
<td>Cerezym®</td>
</tr>
<tr>
<td>Myeloleukemia and other hemoblastoses</td>
<td>Velcade®, Glivec®, MabThera®, Fludara®,</td>
</tr>
<tr>
<td>Multiple sclerosis</td>
<td>Copaxon-Teva®, Rebif®, Betaferon®</td>
</tr>
<tr>
<td>Condition after organ and (or) tissue transplant</td>
<td>Myfortic®, Cellsept®, Prograf®, Sandimune®</td>
</tr>
</tbody>
</table>
State program of pharmacological support of patients with 7 rare and expensive diseases (2008)

<table>
<thead>
<tr>
<th>Diseases</th>
<th>Total of patients</th>
<th>Total sum, millions €</th>
</tr>
</thead>
<tbody>
<tr>
<td>Hemophilia</td>
<td>7109</td>
<td>258</td>
</tr>
<tr>
<td>Mucoviscidosis</td>
<td>1380</td>
<td>18</td>
</tr>
<tr>
<td>Pituitary nanism</td>
<td>2254</td>
<td>14</td>
</tr>
<tr>
<td>Gaucher disease</td>
<td>151</td>
<td>33</td>
</tr>
<tr>
<td>Myeloleukemia and other hemoblastoses</td>
<td>13583</td>
<td>357</td>
</tr>
<tr>
<td>Multiple sclerosis</td>
<td>11328</td>
<td>157</td>
</tr>
<tr>
<td>Condition after organ and (or) tissue transplantation</td>
<td>10088</td>
<td>43</td>
</tr>
<tr>
<td><strong>Total:</strong></td>
<td><strong>45893</strong></td>
<td><strong>880</strong></td>
</tr>
</tbody>
</table>
Strategies for rare diseases

- **Laws regarding RDs are absent**
- **Official definition of RDs in Russia is absent**
- **There is definition by Formulary Committee of Russian Academy of Medical Sciences “rarely used medical technology” (2006)**

Rarely used medical technologies:

- must have high level of evidence of the effectiveness;
- must have vital necessity (without its administration in life-threatening diseases and syndromes progression of the disease or deterioration of its coarse, complications or patient’s death may develop);
- must rarely used (real or forecasting) – **in less than 10,000 people in Russian Federation**
Strategies for rare diseases

List of rarely used medical technologies of Formulary Committee of Russian Academy of Medical Sciences (November 2008)

- 52 treatment drugs;
- 2 diagnostic drugs (corticotropin releasing hormone, filter strip for “dry” eye diagnostics;
- 3 treatment method (LDL apheresis, Lp(a) apheresis, Ig apheresis)

<table>
<thead>
<tr>
<th>European marketing authorized Orphan Drugs (October 2008)</th>
<th>From them authorized in Russian Federation</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>All</td>
</tr>
<tr>
<td></td>
<td>50</td>
</tr>
<tr>
<td></td>
<td></td>
</tr>
</tbody>
</table>
Funding of actions, regarding rare diseases

- **Prenatal screening** (ultrasonic diagnostics, biochemical diagnostics – alfa-fetoprotein, chorionic gonadotropin, cytogenetic diagnostics)
- **Neonatal screening** within the National project «Health» (fenilketonuria, congenital hypothyroidism, galactosemia, adrenogenital syndrome, mucoviscidosis)
- **Diagnostic genetic services** (regional medico-genetic consultations – possibilities are very limited except Moskow, St.Petersburg, Tomsk and several large cities)
- **Treatment (clinical management)** – several clinics in Moskow in St.Petersburg, genetic clinic in Tomsk
- **Orphan drugs** (official definition is absent)
- **Rehabilitation** (only for invalids)
- **Social care** (only for invalids and children-orphans)
Provision of information for rare diseases

The information for rare diseases in Russian is very limited

• **Website-based information** for rare diseases in Russian is very limited
  – [www.rarediseases.ru](http://www.rarediseases.ru) – Information portal for RDs, orphan drugs and rarely used medical technologies (the portal has started to work in January 2009)
  – [redkieboleznii.narod.ru](http://redkieboleznii.narod.ru) – Rare diseases – Database for RDs (the site has started to work in 2009, while 15 diseases)
  – [www.rspor.ru/ps/](http://www.rspor.ru/ps/) – Professional service for rare expensive diseases of Formulary Committee of Russian Academy of Medical Sciences (only 7 expensive diseases)

• **Help Lines** – Hot line of National Association of Organization of Patients with Rare Diseases «Genetics» (the hot line has started to work in September 2008)

• **Official list of RDs is absent.**
RAREDISEASES.RU - информационный портал по редким заболеваниям, "лекарствам - сиротам" и редко применяемым медицинским технологиям

ВАЖНО!

Горячая линия Ассоциации «Генетика» 8(812) 319-34-23

Информация на этом сайте предоставляется бесплатно для образовательных целей и не может быть использована для самодиагностики и лечения. В случае возникновения проблем со здоровьем, пожалуйста, проконсультируйтесь с врачом!

Уважаемый посетитель!

В настоящее время, несмотря на развитие Интернета и другие революционные достижения в области информационных технологий, существует огромный дефицит информации, особенно русскоязычной, о редких заболеваниях. Пациенту обычно не хватает информации о современных возможностях диагностики и лечения его заболевания, рекомендаций по изменению образа жизни, сведений об организациях оказывающих поддержку. Практический врач часто не знает особенностей диагностики и лечения редкого заболевания, не знает, куда направить пациента для оказания необходимой помощи. Ваш сайт может стать единственным источником информации, который может помочь пациенту.
Research on rare diseases

• State research programme for RDs and coordination of researches while is absent.
• Register of researches for RDs is absent.
• There are local research programmes in several scientific research institutes, universities and academies (Research Centre for Medical Genetics Russian Academy of Medical Sciences, Moskow; Research Institute of Medical Genetics TSC Russian Academy of Medical Sciences, Tomsk etc.)
Prevention

- **Folic acid** (official recommendations are absent, is applied at discretion of physician)
- **Prenatal screening** (ultrasonic diagnostics, biochemical diagnostics – alfa-fetoprotein, chorionic gonadotropin, cytogenetic diagnostics)
- **Neonatal screening** (only fenilketonuria, congenital hypothyroidism, galactosemia, adrenogenital syndrome, mucoviscidosis)
- **Delay of diagnosis** (very often in several large cities, in regions diagnostics can be absent)
Type of patients organizations

– The big organizations with the wide experience
  • All-Russia charitable public organization of invalids “All-Russian society of hemophilia” (formed in 2000,
  • Multiregional public organization “Care of patients with mucoviscidosis” (formed in 1997)

– The small organizations
  • Multiregional charitable public organization “Society of invalids suffering from Hunter’s syndrome, other forms of mucopolysaccharidosis and another rare genetic diseases” (created in 2004),

– The umbrella-organizations
  • National Association of organization of patients with rare diseases «Genetics» (formed in 2008)

– Organizations were headed by chief executive paid by pharmaceutical firm
– Organizations were headed by physicians
– Organizations were headed by patients
Empowerment of patients organizations

• **Support to the activities of patient organizations**
  – The government support is only for invalid’s organizations
  – The non-government support (NGO, pharmaceutical firm etc.) is for non-invalid’s organizations

• **Representation and consultation of patient organizations**
  – Consulting non-government organizations (legal consultation and help)
  – Umbrella-organizations (National Association of patients with rare diseases «Genetics»)
Specialised social services

- **Care Services** – only for invalids
- **Therapeutic Recreational Programmes** – only for invalids
- **Services aimed at the integration of patients in daily life** is absent (there are the first steps of patient’s organizations in this area)
Russia: more questions than answers

- Absence of relevant register of patients with rare diseases (medical archives are not a register)
- Absence of register of medical clinics with conditions for diagnosis and treatment of such diseases and specialists with enough experience in this area.
- Insufficiency of quality and available information and scientific knowledge about rare diseases.
- Limitation or absence of possibilities for diagnosis of the majority of rare diseases.
- Difficulties in gaining access to treatment if it exists.
- Absence of protocols of control of patients in the majority of the diseases.
Russia: more questions than answers

- Absence of education programs for doctors of polyclinics in rare diseases.
- In absence of diagnosis, registration and protocols of control of patients there is no basis for appropriation of budgetary funds for their treatment.
- Insufficiently effective and inadaptable system of drug assurance.
- Absence of laws regulating situation with rare disease and drugs for their treatment.
- Absence of working system of planning and adaptable monitoring of situation with rare diseases.
- Incomplete mutual understanding, interaction and coordination in public authorities, medical and social workers and patients.
These problems are not individual, they are shared problems for the majority of countries. Difference is in that in other countries these problems have been already started to be resolved, in Russia this problem doesn’t exist from the viewpoint of civil servants.