Economy of rare (orphan) technologies

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In the world

- Theme of rare (orphan) technologies appeared in USA in 1983
- In Europe there are pan-European documents
- European List of Orphan Drugs has 440 positions
Main groups of orphan medical technologies

- Hereditary diseases of cell’s metabolism
- Central nervous system diseases
- Hematology
- Oncology
- Thrombophilias
- Transplantology
- Cardiovascular, lung and mixed diseases
- Rheumatic diseases
In Russia

• List of orphan medical technologies appeared in 2005
• Nowadays it contains 40 technologies
• There is no special legislation and regulation in Russia
Orphan diseases and orphan technologies

- Affect small number of people – from several to 10 000 in Russia
- There is a treatment with a dramatic effect available
- Target therapy
- Changes in diseases classifications
- There is need for different diagnostic approach
- Rarity makes it impossible:
  - To conduct common clinical studies
  - To register across the world
  - Sell from drugstores
  - Sell for prices, based on a prime cost
Patients’ registry – new methodology of investigation becomes available

- Investigating “typical practice” or “real world”
- There is a need for simple, automatic, standardized process. Both doctors and patients take part in the development and management of registries
- Time-consuming (for rare diseases)
- Should be international (especially for rare diseases), always multicentered
- Can lead to unexpected results, that contradicts to existing stereotypes and practice
- Should include economics issues
Antibodies to antihemophilic factor VIII as an example of orphan technologies

- Patients appeared after introducing of VIII factor in 2005 (10-times increasing consumption, in 2008 2.5-times increasing)
- Number of patients with antibodies – 150 people, should be about 600
- Antibodies are detected only in 4-5 regions of Russia
- Cost of treatment:
  - To stop bleeding - 120 000 USD per year for the whole life
  - To remove antibodies - up to 8 mln. USD per year, then – standard treatment (not always helps!)
Issues of orphan diseases diagnosing

- High-specialized molecular-biologic methods – expensive equipment, trained personnel
- In the world – diagnostics is concentrated in specialized centres, in Russia – is made by pharmaceutical companies
- There is no practice of obtaining and post delivery of biologic material
- There is neither scientific nor managing program of development
Professional Service for Rare (Orphan) Diseases of Formulary Committee of Russian Academy of Medical Sciences

- Standards and protocols of medical care
- Public monitoring of governmental program of drug supply for 7 rare diseases
- Proposals for regulating documents
- Collaboration of different target groups – doctors, patients, manufactures, distributors, managers
- Assessment of quality of life and economics of program
- Creation of state policy and governmental program for rare diseases, including diagnostic issues
Orphan diseases and orphan drugs

• **Hypophysial nanism**: Somatropin
• **Multiple sclerosis**: Glatiramer acetate, Interferon beta-1a, Interferon beta-1b
• **Myeloid leukemia and other hemoblastosis**: Bortezomib, Imatinib, Rituximab, Fludarabine
• **Gaucher's disease**: Imiglucerase
• **Cystic fibrosis**: Dornase alfa
• **Transplantation**: Mycophenolic acid, Mycophenolate mofetil, Tacrolimus, Ciclosporin
• **Hemophilia**: Antihemophilic factor VIII, Octocog alfa, Antihemophilic factor IX, Eptacog alfa (activated)
Распоряжение Правительства РФ от 2 октября 2007 г. №1328-р – об утверждении перечня централизованно закупаемых за счет федерального бюджета ЛС, предназначенных для лечения «дорогостоящих» заболеваний.

Постановление Правительства РФ от 17 октября 2007 г. № 682 – о централизованной закупке в 2008 и 2009 гг. лекарственных средств, предназначенных для лечения больных гемофилией, муковисцидозом, гипофизарным нанизмом, болезнью Гоше, миелолейкозом, рассеянным склерозом, а также после трансплантации органов и (или) тканей.

Приказ Минздравсоцразвития РФ от 19 октября 2007 г. № 650 – о формах заявок на поставку ЛС.

Приказ Минздравсоцразвития РФ от 28 ноября 2007 г. № 727 – об органе, осуществляющем ведение Федерального регистра больных.

Постановление Правительства РФ от 4 декабря 2007 г. № 840 – о предоставлении субсидий из федерального бюджета бюджетам субъектов РФ на осуществлении организационных мероприятий по обеспечению граждан ЛС.
### Number of patients with 7 orphan diseases (Federal Agency for Health Care and Social Development, January 1, 2008)

<table>
<thead>
<tr>
<th>Disease</th>
<th>Federal privileged persons</th>
<th>Regional privileged persons</th>
<th>Total</th>
</tr>
</thead>
<tbody>
<tr>
<td>Hemophilia</td>
<td>5733</td>
<td>1376</td>
<td>7109</td>
</tr>
<tr>
<td>Cystic fibrosis</td>
<td>1313</td>
<td>67</td>
<td>1380</td>
</tr>
<tr>
<td>Hypophysial nanism</td>
<td>1474</td>
<td>780</td>
<td>2254</td>
</tr>
<tr>
<td>Gaucher’s disease</td>
<td>119</td>
<td>32</td>
<td>151</td>
</tr>
<tr>
<td>Myeloleukemia and other hemoblastosis</td>
<td>11935</td>
<td>1648</td>
<td>13583</td>
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<tr>
<td>Multiple sclerosis</td>
<td>9272</td>
<td>2056</td>
<td>11328</td>
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<tr>
<td>Transplantation</td>
<td>9698</td>
<td>390</td>
<td>10088</td>
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<tr>
<td><strong>TOTAL:</strong></td>
<td><strong>39544</strong></td>
<td><strong>6349</strong></td>
<td><strong>45893</strong></td>
</tr>
</tbody>
</table>
### Volume of purchase (1st and 2d half years 2008)

*(Federal Agency for Health Care and Social Development, March 2008)*

<table>
<thead>
<tr>
<th>Disease</th>
<th>Volume of purchase (RUB.)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Hemophilia</td>
<td>3 969 670 814,62</td>
</tr>
<tr>
<td></td>
<td>5 049 364 646,85</td>
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<tr>
<td>Cystic fibrosis</td>
<td>271 497 655,17</td>
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<td></td>
<td>344 961 028,93</td>
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<tr>
<td>Hypophysial nanism</td>
<td>449 937 989,52</td>
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<tr>
<td></td>
<td>48 932 337,65???</td>
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<tr>
<td>Gaucher’s disease</td>
<td>575 466 740,14</td>
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<tr>
<td></td>
<td>564 851 929,29</td>
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<tr>
<td>Myeloleukemia and other hemoblastosis</td>
<td>6 721 133 408,64</td>
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<tr>
<td></td>
<td>5 760 122 684,81</td>
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<tr>
<td>Multiple sclerosis</td>
<td>271 497 655,17</td>
</tr>
<tr>
<td></td>
<td>2 791 076 199,21 ???</td>
</tr>
<tr>
<td>Transplantation</td>
<td>658 481 380,41</td>
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<tr>
<td></td>
<td>861 505 599,09</td>
</tr>
<tr>
<td><strong>TOTAL:</strong></td>
<td>15 359 648 197,87 ???</td>
</tr>
<tr>
<td>There is lack of coincidence in official data</td>
<td>15 767 699 672,85 ???</td>
</tr>
</tbody>
</table>

There is lack of coincidence in official data.
Expensive drugs delivery in 2006 – 2008 (mln. RUB)
(Federal Agency for Health Care and Social Development, March 2008)

We have other data
Main challenges

• To investigate quality of life in patients suffering from 7 orphan diseases
• To investigate correlation of quality of life with treatment
• To determine cost of QALY
Governmental program for orphan diseases

- Diagnostic in diagnostic centres for orphan diseases (3-4 across Russia)
- Centres for orphan diseases in all regions of Russia
- Responsible for orphan diseases doctors in each region and territory
- Address delivery of drugs
- Home treatment
- Centres for drug infusion and injection in one-day hospitals
- Registers with participation of primary care doctors