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ПРОЕКТИ НА БЪЛГАРСКА АСОЦИАЦИЯ ЗА ПРОМОЦИЯ НА ОБРАЗОВАНИЕ И НАУКА (БАПОН)

Issue 3

September 2012

# ORPHAN DRUGS IN BULGARIA

*PERIODIC REVIEW OF THE ACCESS TO ORPHAN DRUGS IN BULGARIA*

## **Methodology**

Orphan drug is a medical product if its sponsor can establish:

- (a) that it is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10 thousand persons in the Community when the application is made, or that it is intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition in the Community and that without incentives it is unlikely that the marketing of the medicinal product in the Community would generate sufficient return to justify the necessary investment;
- (b) and that there exists no satisfactory method of diagnosis, prevention or treatment of the condition in question that has been authorised in the Community or, if such method exists, that the medicinal product will be of significant benefit to those affected by that condition.

The orphan drugs, included in this report, have been designated under the Regulation (EC) No 141/2000 and have marketing authorisation and positive evaluation of significant benefits.

The list is arranged by tradename in alphabetical order, including active substance and indications, as well information about the presence of the medicine in the Positive drug list (PDL) of Bulgaria.

Used sources and links:

- European medicines agency (EMA) – [Registry of human medicines](#)
- Ministry of health (MoH) – [Positive drug list](#)

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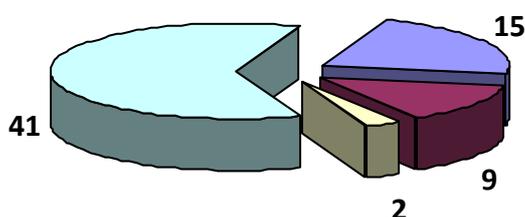
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## SUMMARY

ICRDOD continues its review series, discussing various aspects of rare diseases in Bulgaria. A year and a half since the last review of this particular topic (March 2011), we return to the access to orphan drugs. At the end of 2011, there were major changes in the national legal framework on this issue. As of September 2012 the list of available and accessible orphan drugs in Bulgaria is as follows:

Trade name	Active substance	Indication	PDL
Atriance	nelarabine	T-cell acute lymphoblastic leukaemia, T-cell lymphoblastic lymphoma	A2
Cayston	aztreonam lysine	Cystic fibrosis	A2*
Elaprase	idursulfase	Mucopolysaccharidosis type II	A1, A2
Evoltra	clofarabine	Acute lymphoblastic leukemia	A2
Exjade	deferasirox	Beta thalassemia major	A1, A2
Fabrazyme	agalsidase beta	Fabry disease	A1
Glivec	imatinib	Ph+ chronic myeloid leukemia, Ph+ acute lymphoblastic leukemia, myelodysplastic/myeloproliferative diseases, advanced hypereosinophilic syndrome and/or chronic eosinophilic leukemia, gastrointestinal stromal tumours	A1, A2
Litak	cladribine	Hairy cell leukemia	A2
Lysodren	mitotane	Advanced adrenal cortical carcinoma	A2
Mozobil	plerixafor	Collection of haematopoietic stem cells to the peripheral blood for transplantation in patients with lymphoma and multiple myeloma	A2
Nexavar	sorafenib	Hepatocellular carcinoma, advanced renal cell carcinoma	A1, A2
Nplate	romiplostim	Chronic immune thrombocytopenic purpura	A2
Revatio	sildenafil	Pulmonary arterial hypertension	A1
Revolade	eltrombopag	Chronic immune thrombocytopenic purpura	A1, A2
Somavert	pegvisomant	Acromegaly	A1, A2
Sprycel	dasatinib	Ph+ chronic myeloid leukemia, Ph+ acute lymphoblastic leukemia	A1, A2
Tasigna	nilotinib	Ph+ chronic myeloid leukemia	A1, A2
Tobi Podhaler	tobramycin	Cystic fibrosis	A1, A2*
Torisel	temsirolimus	Advanced renal-cell carcinoma, mantle-cell lymphoma	A2
Tracleer	bosentan monohydrate	Pulmonary arterial hypertension, scleroderma	A1
Ventavis	iloprost	Pulmonary arterial hypertension	A1, A2
Volibris	ambrisentan	Pulmonary arterial hypertension	A1, A2
Votubia	everolimus	Subependymal giant cell astrocytoma	A1, A2
Xagrid	anagrelide	Essential thrombocythaemia	A2
Yondelis	trabectedin	Advanced soft tissue sarcoma, ovarian cancer	A2
Zavesca	miglustat	Gaucher disease type 1, Niemann-Pick type C disease	A1, A2

\* In the Positive Drug List of Bulgaria (PDL) is included a medicinal product with the same active substance, but in a different medicinal form than the one in the EMA authorisation decision.



- Accessible (reimbursed by NHIF)
- Accessible (reimbursed by hospital budgets)
- Included in PDL under different form
- Inaccessible in Bulgaria

Currently, the European Medicines Agency (EMA) has authorised 67 orphan drugs for use. In Bulgaria, two thirds of them are practically inaccessible due to the fact that they are not reimbursed. You can find more on the reasons for that in ICRDOD paper [Challenges to orphan drugs access: the case of Bulgaria](#), which was recently published in the prestigious international journal *Health Policy*.