



Expert Review of Clinical Pharmacology >

Volume 5, 2012 - [Issue 5](#)

53 | 11
Views | CrossRef citations to date | Altmetric

Editorial

Medicines for pediatric oncology: can we overcome the failure to deliver?

Agnes Saint-Raymond & Ralf Herold

Pages 493-495 | Published online: 10 Jan 2014

 Cite this article  <https://doi.org/10.1586/ecp.12.51>

Sample our 
Medicine, Dentistry, Nursing
& Allied Health Journals
>> [Sign in here](#) to start your access
to the latest two volumes for 14 days

 Full Article

 Figures & data

 References

 Citations

 Metrics

 Reprints & Permissions

 Read this article

 Share

For reprint orders, please contact reprints@expert-reviews.com

Medicines for pediatric oncology: can we overcome the failure to deliver?

Expert Rev. Clin. Pharmacol. 5(5), 493–495 (2012)



**Agnes
Saint-Raymond**

Author for correspondence:
European Medicines Agency,
7 Westferry Circus, Canary
Wharf, London E14 4HB, UK
Tel.: +44 207 523 7017
Fax: +44 207 523 7040
agnes.saint-raymond@ema.europa.eu



Ralf Herold

European Medicines Agency,
7 Westferry Circus, Canary
Wharf, London E14 4HB, UK

“The way forward regarding pediatric oncology is under discussion by the Committee and may include revoking the condition waivers, giving back to the Committee the possibility to analyze the mechanism of action and target of the new medicines in order to eventually obtain the necessary data in children within a reasonable timeframe.”

To date, children do not have access to the medicines necessary to treat pediatric cancers. Pediatric tumors have different names and specificities from adult tumors that go far beyond the naming issue. In most other therapeutic areas, with the main exception in pediatric rheumatology, the diseases affecting children are close to those affecting adults with respect to type of diseases and pathophysiology. Owing to specific gene mutation and expression profiles and fast tumor growth, cancers affecting children are different. An aggravating factor for the lack of authorized medicines is the rarity of the diseases, representing a small market overall, although they are frequent among serious pediatric diseases. Cancer represents the second main cause of death in children and treatments place a heavy burden on the child and his or her family. The uncertainties on long-term prognosis owing to potential late relapses, as well as complications of chemotherapy and radiotherapy add to this burden. However, pediatric oncology is also the area where treatments have achieved outstanding results through rigorous protocols (using medicines off-label), resulting in long-term survival and cures [1].

Another paradox is that most of the treatments used today were established by academics and hospital pediatric oncologists over the last decades, generally with little help or interest from large

pharmaceutical companies. This meant in practice that access to anticancer medicines was always significantly delayed for children [2] in contradiction with ethical principles and existing guidelines such as the International Conference on Harmonization guideline on the development of pediatric medicines (E11), which requires simultaneous submission for adults and children when the disease is serious or life threatening, when there are limited or no therapeutic alternatives [10].

In fact, behind the success lies another reality. If most of the success comes from effective treatments of acute pediatric leukemia and lymphoma, many other tumors – especially advanced stages and those of the CNS such as high-grade glioma – remain without effective therapeutic options, with short survival and devastating effects on the child and the family [3].

Regulatory initiatives in the USA, then in Europe, aim to ensure that medicines intended for adults are developed for children where there are unmet pediatric needs [102,103]. Unfortunately, to date in pediatric oncology these have not been so successful for several reasons. As part of the US Best Pharmaceutical for Children Act (BPCA), the US FDA can issue Written Requests based on public health needs describing how a company can develop a medicine for children, with the prospect of getting additional

KEYWORDS: child • development • medicines • oncology • regulation

**EXPERT
REVIEWS**

Disclaimer

The views presented in this editorial are those of the author and should not be understood or quoted as being made on behalf of the EMA and/or its scientific Committees.

Financial & competing interests disclosure

The authors have no relevant affiliations or financial involvement with any organization or entity with a financial interest in or financial conflict with the subject matter or materials discussed in the manuscript. This includes employment, consultancies,

honoraria, stock ownership or options, expert testimony, grants or patents received or pending, or royalties.

No writing assistance was utilized in the production of this manuscript.

Log in via your institution

➤ [Access through your institution](#)

Log in to Taylor & Francis Online

➤ [Log in](#)

Restore content access

➤ [Restore content access for purchases made as guest](#)

Purchase options *

[Save for later](#)

PDF download + Online access

- 48 hours access to article PDF & online version
- Article PDF can be downloaded
- Article PDF can be printed

EUR 92.00

 [Add to cart](#)

Issue Purchase

- 30 days online access to complete issue
- Article PDFs can be downloaded
- Article PDFs can be printed

EUR 249.00

 [Add to cart](#)

* Local tax will be added as applicable

Related Research

People also read

Recommended articles

Cited by
11

Information for

Authors

R&D professionals

Editors

Librarians

Societies

Opportunities

Reprints and e-prints

Advertising solutions

Accelerated publication

Corporate access solutions

Open access

Overview

Open journals

Open Select

Dove Medical Press

F1000Research

Help and information

Help and contact

Newsroom

All journals

Books

Keep up to date

Register to receive personalised research and resources
by email

 Sign me up

