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Editorial

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Editorial

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

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"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 [101].

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

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EXPERT REVIEWS



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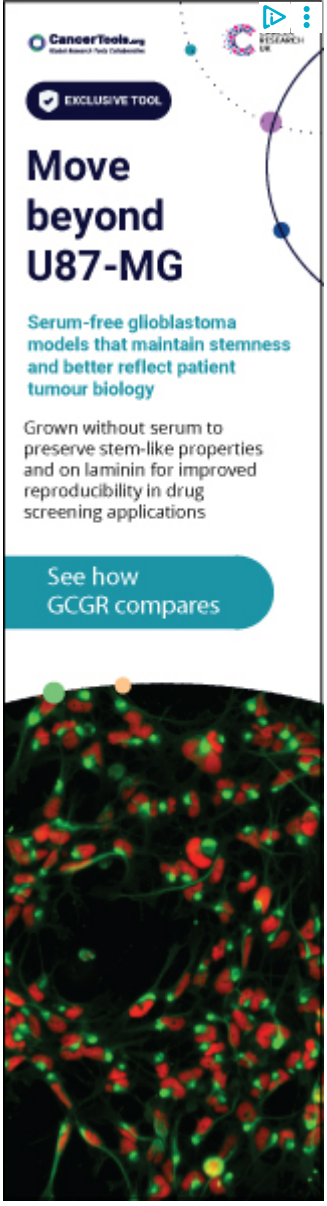


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