

CCBIO Opinion
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Precision Medicine - Fairness and the Big Pharma Perspective

The days of the Blockbuster drug are over in oncology. The requirement to demonstrate convincing benefit for patients for a new cancer drug in order to license it is driving increasingly rigorous patient stratification as a means to unambiguously demonstrate precise patient cohorts in whom treatment is justified. Big pharma understand that these cohorts are increasingly precisely molecularly stratified, and in general there is an accompanying push to use new drugs earlier in the disease journey as this trends to an increase in seeing benefit. Thus, big science in big pharma, richer nations' health budgets and insurance systems that can cope with this complexity and therefore spiraling costs, together with the ability to present cogent cases for novel treatments in increasingly sophisticated ways result in the more affluent microcosmos of our societies benefiting first from iconoclastic approaches, in contrast to far more restrictive approaches, if any

access at all, from poorer public health care systems earlier in the life cycle of new drugs. The recent examples of the eye-watering cost associated with FDA approval of CD19 targeted Chimeric Antigen Receptor T Cell therapy for relapsed paediatric acute lymphoblastic leukemia is no doubt based on the hope of future quality of life and productivity for that child together with the relative rarity of the condition.

The typical patient utilised en-route to registration of new anti cancer drugs is not typical of the general population of cancer patients and clearly demographic and socioeconomic issues contribute to that difference (awareness through education, nutrition, earlier presentation etc). Nevertheless, big pharma has a responsibility to follow the science and turn that science into good medicine, inevitably creating an environment where some patients will benefit disproportionately through serendipity

born out of personalisation in addition to better access through socioeconomics. The successes of precision medicine are creating a complex mosaic of trade-offs where fairness and equity of access to life lengthening or curative but expensive treatments have to be scrutinised. This of course has a "reverse translational" effect on the way drug development decisions are made in pharma, and the symbiosis of big pharma and society continuously evolves around these issues.

Despite these caveats I remain optimistic that good science will give us solutions to disease both preventative and curative, and that big pharma has an essential role in creating the landscape of clinically available options that can create the debate around fairness through an embarrassment of riches rather than desert of hopelessness. ••

