

# **Fair, just and compassionate: A pilot for making allocation decisions for patients requesting experimental drugs outside of clinical trials**

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## **Abstract**

Patients have received experimental pharmaceuticals outside of clinical trials for decades. There are no industry-wide best practices, and many companies that have granted compassionate use, or 'preapproval', access to their investigational products have done so without fanfare and without divulging the process or grounds on which decisions were made. The number of compassionate use requests has increased over time. Driving the demand are new treatments for serious unmet medical needs; patient advocacy groups pressing for access to emerging treatments; internet platforms enabling broad awareness of compelling cases or novel drugs and a lack of trust among some that the pharmaceutical industry and/or the FDA have patients' best interests in mind. High-profile cases in the media have highlighted the gap between patient expectations for compassionate use and company utilisation of fair processes to adjudicate requests. With many pharmaceutical manufacturers, patient groups, healthcare providers and policy analysts unhappy with the inequities of the status quo, fairer and more ethical management of compassionate use requests was needed. This paper reports on a novel collaboration between a pharmaceutical company and an academic medical ethics department that led to the formation of the Compassionate Use Advisory Committee (CompAC). Comprising medical experts, bioethicists and patient representatives, CompAC established an ethical framework for the allocation of a scarce investigational oncology agent to single patients requesting non-trial access. This is the first account of how the committee was formed and how it built an ethical framework and put it into practice.

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