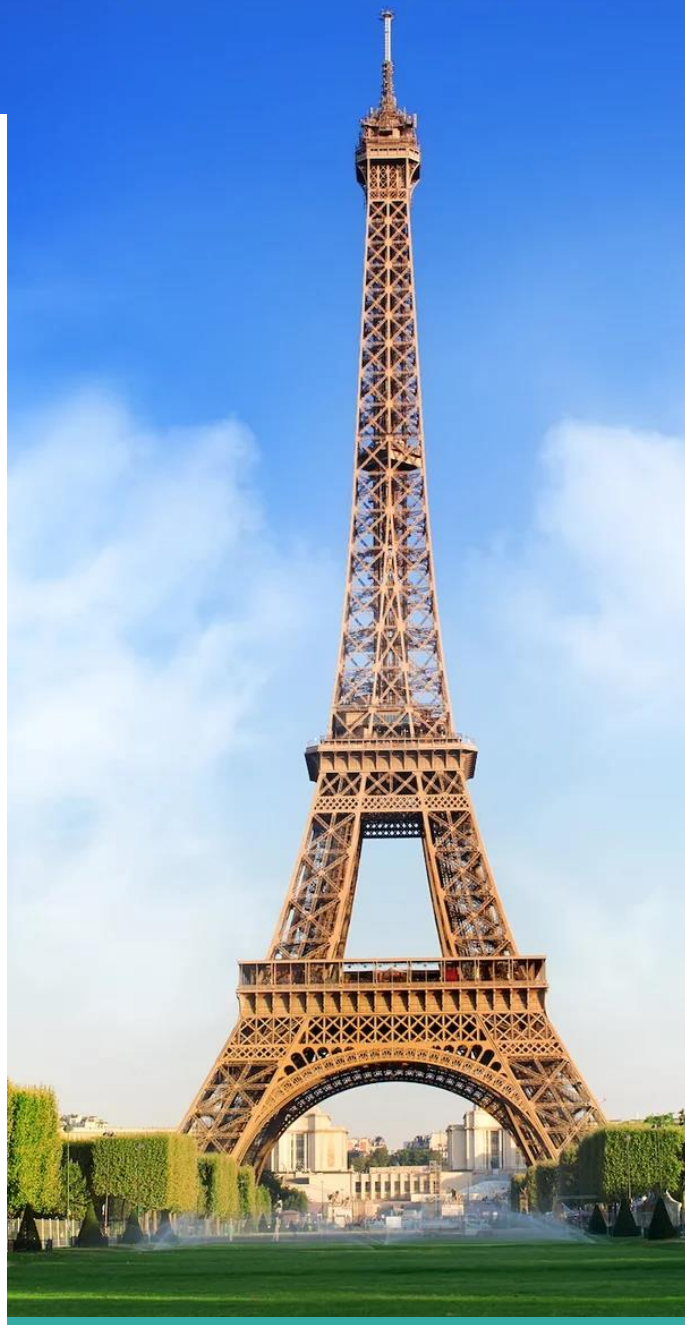
An orphan drug perspective on the new French early access framework: so long ATU, welcome early access!



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Early Access Programs (EAPs) - known as Expanded Access Programs in the US - allow pre-approval access to medicines for certain patients. These EAPs provide pre-launch access for drugs in advance of their marketing authorization for patients with life-threatening conditions where there are no approved treatment options available. EAPs are country-specific, indeed, patient access on a national level remains very heterogeneous across Europe due to different healthcare organizations.

Created in France in the early 90's[1] as part of the fight against AIDS, the ATU (Temporary Use Authorization system) has enabled France to be an anticipated access pioneer to innovative drugs in Europe. The current French system, progressively made up of an accumulation of different mechanisms and safeguards such as post-ATU or RTUs (Temporary Recommendations for Use), has become very complex. To tackle this matter, LFSS (The French Social Security Financing Law) for 2021[3] introduced article 78, that aims to clarify the derogatory early access program. This article adjusts all these mechanisms (cohort, nominative, post-ATU, RTU) into two distinct access structures that will become applicable by 1st July 2021 at the latest.

On the one hand, the "compassionate access" mechanism will target drugs for which no development in this indication is considered but which meet an unmet therapeutic need. A request for authorization can be made by the prescriber for a nominative treatment for a period of one year (former nominative ATU), or a framework can be carried out at the initiative of the ANSM (French National Security Agency of Medicines and Health Products) for 3 years (former RTU)[4].

On the other hand, the early access mechanism unifies five existing schemes into one: it is granted for a drug in a therapeutic indication for which it is presumed to be innovative under the condition of a commitment by the laboratory to file a marketing authorization application within a given period. The early access program is defined as "the use, on an exceptional basis, of certain drugs, in specific therapeutic indications, intended to treat rare or disabling diseases". The interest of this program for orphan drugs is crucial. Indeed, medicines used in this scope are reimbursed by social security based on a price declared by the exploiting company, making this program very attractive. As a sign of the ATU system effectiveness in providing early access to new therapies, the amount of expenditure on orphan drugs under/post-ATU doubled between 2015 and 2016, from €82 million to €207 million[5]. This spending continued to increase in 2017 reaching €311 million[6]. In 2019, hospital sales (including retrocession, ATU / Post ATU status) represented 70%of orphan drug sales (+38,2% compared to 2018).

Finally, not only is this reform attractive to pharmaceutical companies, but it also addresses one of the commitments made by the French Government in 2019 at the 8th CSIS[2] : to guarantee rapid access to innovation while regaining its European leadership in the life sciences.

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