Webinar

Early Access Programs

17 MARCH 2022

BlueReg
PHARMA CONSULTING

EU Expertise

How to take advantage of early access programs new regulation for your innovative treatments in France ?

Welcome

The speakers

Lucy Pitcher

Senior Regulatory Consultant

Dominique Patrone

General Manager / Vice President
PharmaBlue & Pharmacovigilance

Olivier Roye

Senior Director, Business Development &
Account Management

Webinar Agenda

7 months after the new Early Access Regulation (Replacing ATU)

Why Early Access (AP1/AP2) for new innovative drugs in France is attractive and complex at the same time ?

30min

Why outsourcing your Early Access to a third party “Exploitant” in France is highly recommended ?

Q&A session with the BlueReg experts

15min

Please send your questions for the Q&A using the Q&A box

7 months after the new Early Access Regulation (Replacing ATU)

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Historical « ATU » scheme

- Originally initiated in 1992 for new therapies for HIV
- Enabling the sale of drugs offering solutions to serious or rare diseases ahead of any reimbursement or pricing negotiations
- Since then, it has expanded to cover all areas or research and services

1

Introduction of the procedure into the CSP (Public Health Code)

Article 21 of the Law of 8 December 1992 – Decree of 8 July 1994

2

Introduction of funding in relation to the ATU period

Article 56 of the Law of 20 December 2006

3

Changes to the conditions governing the award of nominative ATUs

Article 26 of the Law of 29 December 2011

4

Perpetuation of funding for medicinal products in the post-MA period & obligation to ensure continuity of ongoing treatment

Article 48 of the Law of 23 December 2013

5

Post-ATU funding by indication & €10k cap

Article 97 of the Law of 23 December 2016

6

Funding for extensions of indication in relation to the ATU/Post-ATU period via a financial compensation system & temporary post-MA funding

Article 65 of the Law of 22 December 2018

7

Funding arrangements for extensions of indications in relation to the ATU/post-ATU period

Decree 2019-855 of 20 August 2019

8

Clarification of (i) the content of the application to be submitted to the ministers for health and social security, and (ii) the information to be provided by the prescriber to the patient

Ministerial Order of 11 October 2019 implemented by decree 2019-855 of 20 August 2019

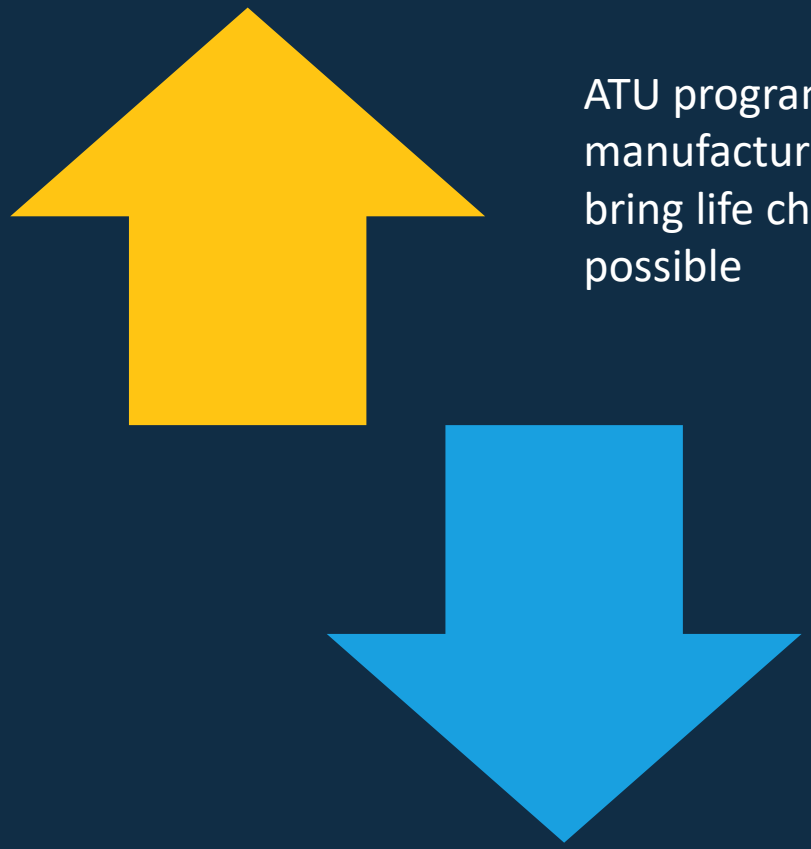
Source: Leem Sep 2021

Important facts about ATU

French regulatory authority (ANSM) recently published a retrospective study on access time for anticancer drugs, comparing FDA, EMA and the French ATU scheme over 13 years:

- Between 1st January 2007 and 31st December 2019, ANSM evaluated and granted ATU in oncology to 36 antineoplastic drugs
- Thanks to the ATU granted, almost 70% (25 out of 36) of drugs were made available in France before FDA approval
- Thanks to ATUs, drugs are, on average available in France 200 days before their first regulatory approval (FDA or EMA)

Complexity of the ATU scheme



ATU programs have been a crucial consideration for manufacturers who seek to minimize access delays and bring life changing therapies to patients as early as possible

This success brought complexity ending with six interlinked schemes, with different perimeters, different eligibility criteria and different reimbursement scheme

Change in regulation as of July 1st, 2021

- Objectives of the reform: consistency – simplicity - clarity
  - Fast access to treatments for patients
  - Reinforce attractiveness to pharma companies
  - Financially sustainable for the healthcare system and pharma companies
- A standardized procedure for all early access applications:
  - Pre-MAA EA application / Post-MAA EA application / Variations / Renewals

Eligibility criteria

- Efficacy and safety are strongly presumed based on clinical data (for pre-MAA early access only)
- Product is indicated for a severe, rare, or incapacitating disease
- No appropriate treatment available
- Initiation of the treatment cannot be deferred
- Product is presumed to be innovative, notably with regards to clinically relevant comparators

Data collection requirements

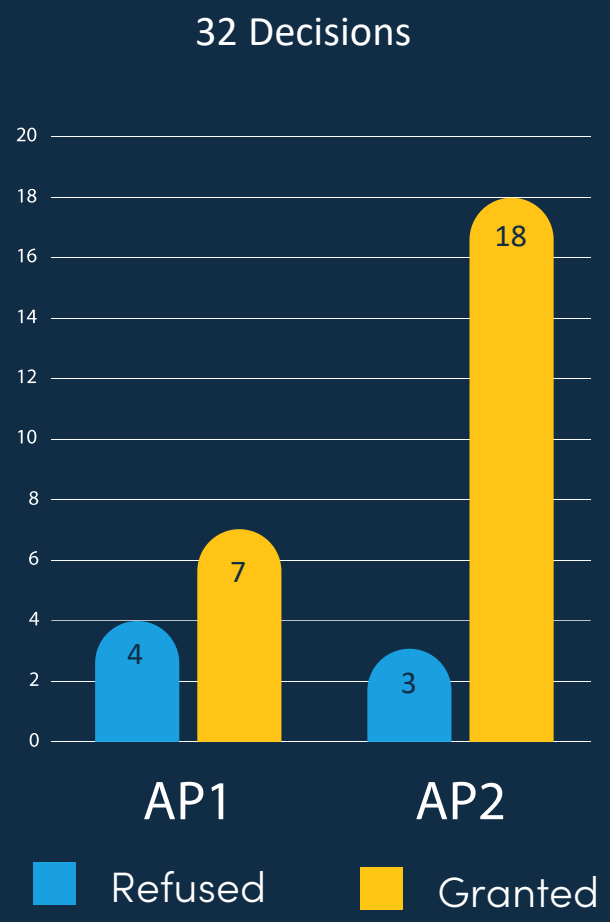
- Data collection defined in a protocol (“PUT-RD”)
- Aiming at capturing data on efficacy, safety, patient characteristics, PRO
- Financing of data collection done by the company
- Collection of at least 90% of data planned required

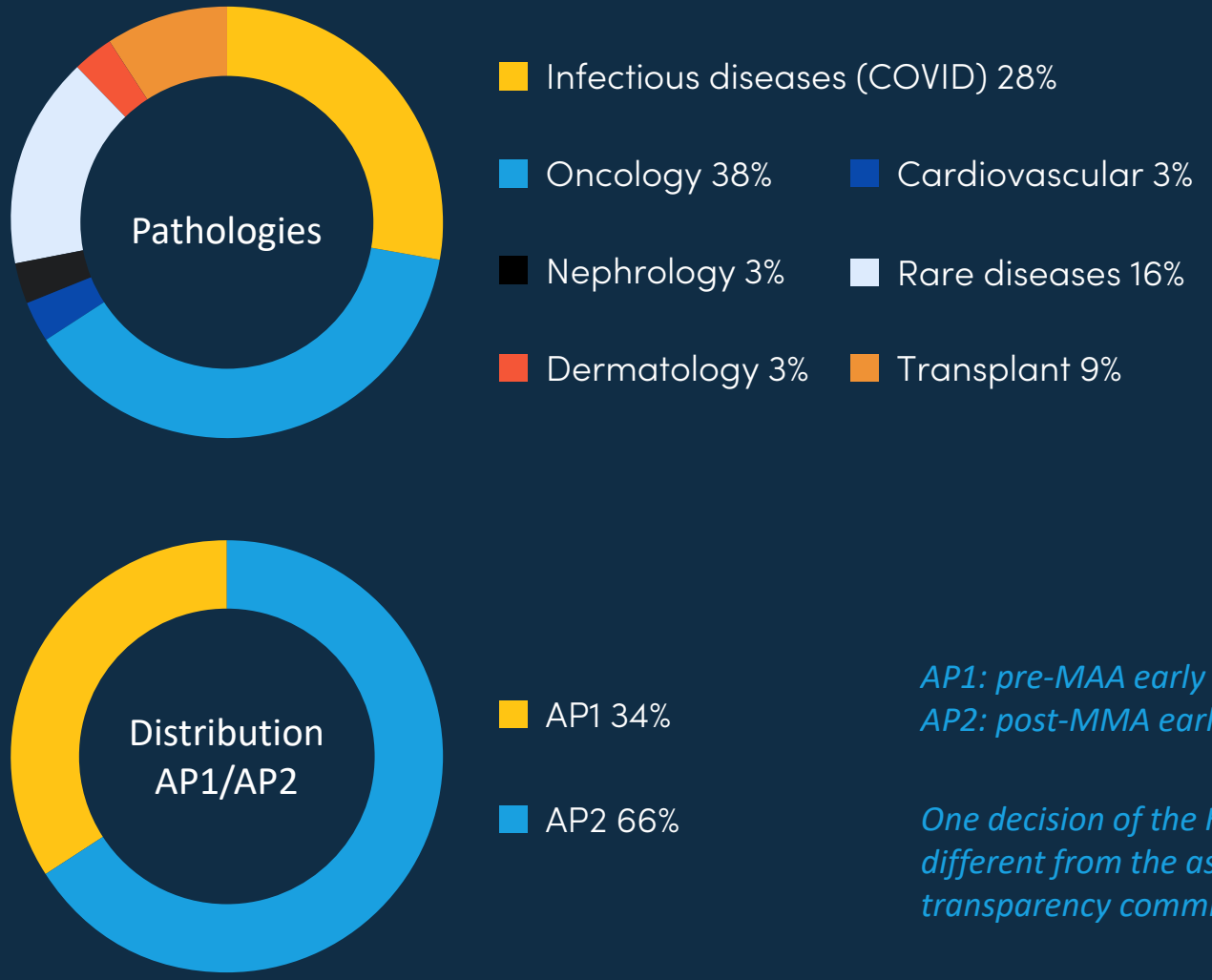
- Recommendations :
  - Quality / completeness of data / compatible with daily clinical practices for HCP
  - Patient involvement in the system, with PRO
  - Use of digital platforms to facilitate data entry
  - Possibility to use of data for future reimbursement dossier / research purposes

Commitments to be made

- Submit a marketing authorization dossier for the indication concerned within 2 years of the EA application
- Apply for reimbursement for the indication concerned within 1 month after MA has been granted
- Ensure continuity of treatment for the patients included at the end of the EA:
  - 1 year in total
  - 3 months being funded by the French health system

Early Access figures (as of 24 Feb. 22)





AP1: pre-MAA early access
AP2: post-MMA early access

One decision of the HAS college was different from the assessment of the transparency committee

Early Access financial considerations

- Free price setting (unless there is already a reference price available in France) with 100% funding by National Health System
- Annual discounts are due, based on the turnover in the therapeutic indication:
  - Prorated on the length of the early access
  - Specific mark-ups are possible
  - Rebates are set to a maximum / year
- Discounts at the end of the early access:
  - Based on the difference between early access price and final list price, if reimbursed
  - Based on the difference between early access price and a reference price defined by French Economic Committee (“CEPS”), if the product is not reimbursed

Why early access for new innovative drugs in France is attractive and complex at the same time?

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Why Early Access in France is attractive?

- A genuine simplification of early access schemes has been introduced
- Patients can benefit from the treatment ahead of first European regulatory approval
- Your product can access market earlier, preparing for future commercial launch in France and in the rest of Europe
  - Allowing continuous access to your drug for patients
  - Building your company image before commercial launch
- Your product can generate turnover before being approved elsewhere in Europe:
  - Setting the scene for future market access (pricing & reimbursement) of your commercial product
  - Accelerating P&R review process with pre-defined timelines

Why EAP in France is complex?

- A challenging transition from the existing schemes to the new ones
- Process is specific to France and not aligned with other EU countries pathways
- Your product must show compliance to the eligibility criteria for early access
- Daily management of EA is time consuming, with numerous administrative steps

- Access has not been eased with the new regulation with numerous administrative constraints which remain, and new ones created
  - dossier size and preparation time
  - >90% of data to be collected
  - eCRF and ePRO tools
  - Various commitments to be made by applicants

Why having a third party to run your EAP in France is highly recommended?

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Why having a third party to run your EAP in France is highly recommended?

- Regulatory Constraints:

  French regulatory authorities require having an « Exploitant » authorized and established in France to manage the EAP

- Financial / Resources Constraints:

  Tangible investment if you want to setup your French affiliate, particularly so early in the access pathway

  Your internal resources are fully dedicated to FDA and/or EMA submission in parallel to EAP request/management

Why having a third party to run your EAP in France is highly recommended?

- Administrative and Management Constraints

Approval process of EAP is specific to France, with pre-submission meetings for pre-license early access and a detailed and standardized dossier to be prepared and submitted

Submission and assessment process requires regular interactions with Authorities

Early Access management requires French speakers to deal with physicians, pharmacists, hospitals, authorities…

PharmaBlue : your solution for EAP management

- PharmaBlue (a BlueReg company) is duly authorized as « Exploitant » by French Regulatory Authorities to operate in France

- With this status, PharmaBlue has managed early access schemes since 2015:
  - We act as your local representative for the authorities
  - We endorse the full « Exploitant » responsibility for your EA:
    - Quality, Distribution, Medical Information, Pharmacovigilance, Communication
  - We can provide support for distribution of your product if needed, through our preferred partner Colca MS (3PL)
  - We can provide and implement qualified tools for data collection, e-CRF

PharmaBlue : your solution for EAP management

Phase 1

We prepare EA dossier as per requirements and guide you through the submission

Phase 2

We manage the review process with authorities until approval, while setting-up all processes for EA program with your teams

Phase 3

We manage EA on your behalf once approved, for you to focus on future commercial launch preparation and EU expansion

As PharmaBlue client, you have also access to BlueReg Group expertise to coordinate your European regulatory compliance for launch activities, promotional material reviews or/and Life cycle management

PharmaBlue : your reference solution for EAP management

Summary

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What have we learned today ?

- Early Access in France is an excellent way :
  - To provide patients with innovative treatment earlier
  - To reach the market earlier (before FDA/EMA approval)
  - To generate revenue & building first reference for pricing
- If you are launching your product in Europe, you will need a partner to manage a smooth transition to a fully operational commercial organization

- “Exploitant” status is mandatory and PharmaBlue provide you with it
- PharmaBlue – Partner for your EA in France from start to end
- PharmaBlue – Partner for the initial steps of your future commercial organization

Q & A Session

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Let’s stay in touch !

- www.blue-reg.com
- contact@blue-reg.com

Thank you !