French Healthcare

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Tuesday, June 15 | 12.30 PM (EST)

Webinar: Early Access Programs in France (ATU)

BIO International Convention DIGITAL

HOSTED BY Bio

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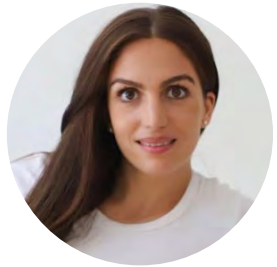
Welcome
The speakers



Elsa Rive
Managing Director, Healthtech
Business France North America

- **Dominique Patrone**
  - General Manager / Vice President PharmaBlue & Pharmacovigilance

- **Olivier Roye**
  - Senior Director, Business Development & Account Management

Welcome
Webinar Agenda

Agenda	Webinar Timings
• Why Early Access (ATU) for new innovative drugs in France is attractive and complex at the same time? • Why having a third party to run your Early Access in France is highly recommended? • Get ready for the new Early Access regulation (Replacing ATU)	30 minutes
• Q&A session with the BlueReg experts	15 minutes

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

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Early Access in France

- An innovative product under development and close to registration might be available for the patients before MAA
- French Authorities display a dedicated process to reach market under specific conditions
- (ATU : Autorisation Temporaire d'Utilisation (Temporary Authorization for Use))
- Will be replaced from July 1st onwards by a new simplified system with 2 pathways: Early Access and Compassionate Use

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

Why ATU in France is attractive?

- 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

ATU in France : Figures & Outcomes- Oncology

- French regulatory authorities (ANSM) recently published* a retrospective study on access time for anticancer drugs, comparing FDA, EMA and the French Early Access (ATU) scheme over 13 years
- What are the main outcomes?
  - 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)

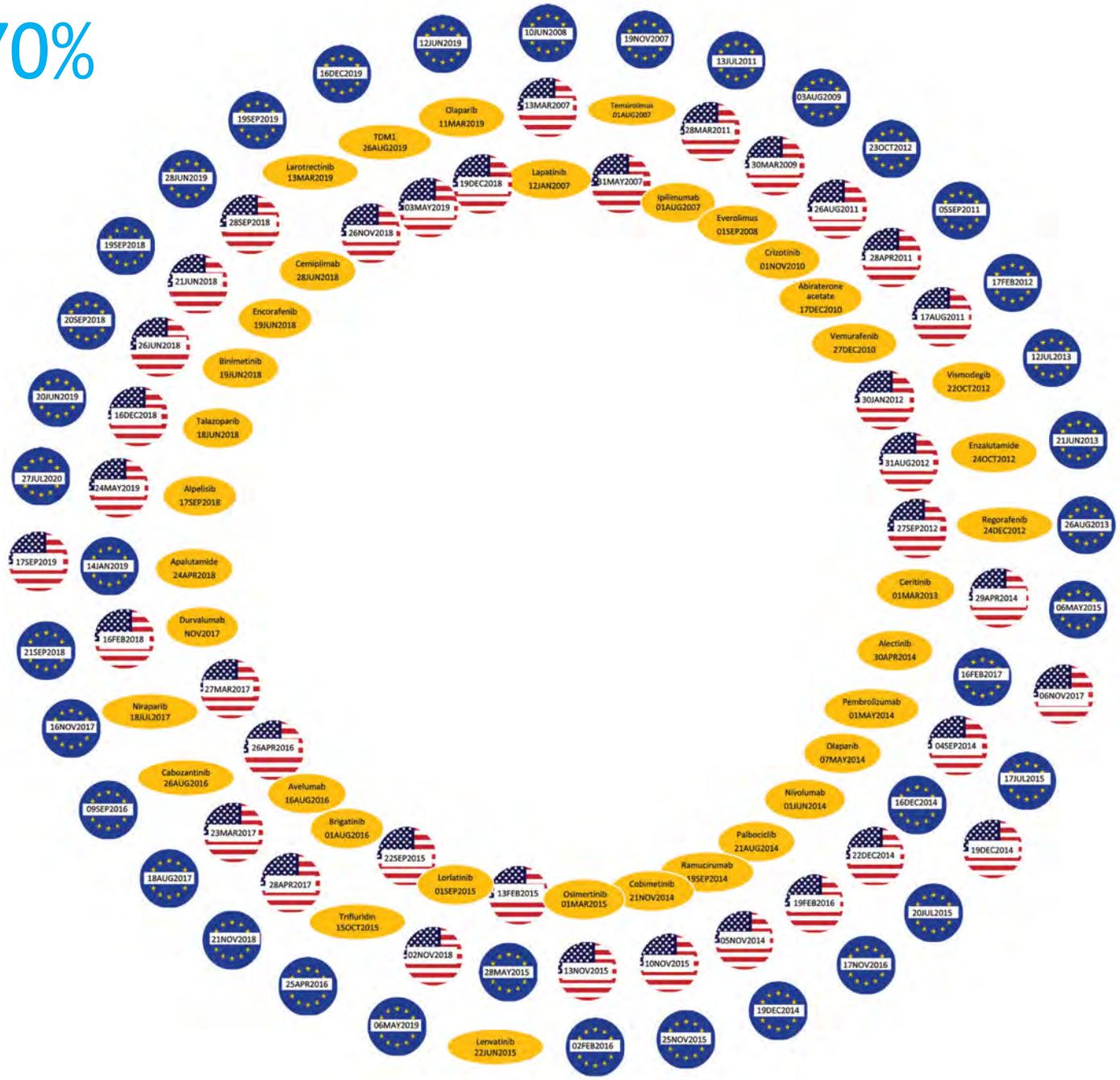
* E. Jacquet et al. European Journal of Cancer 149(2021);82-90

ATU Grant come first in 70% of case

ATU Start Date

FDA approval date

EMA approval date



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Early Access figures in France (ANSM – 2019)

- Number of « cATU's » increased over time but the number of patients was divided by 3
- Generates 720 millions turnover in 2019
- PharmaBlue handles a good part of cATU granted each year

Summary of named-patient ATUs

	2015	2016	2017	2018	2019
Named-patient ATUs granted	24,791	27,095	22,295	21,633	**26,528**
Medicinal products (or active substances) made available per year	219	205	253	217	**227**
Patients included	17,829 including 12,175 treatment initiations	19,625 including 14,029 treatment initiations	16,621 including 11,390 treatment initiations	15,987 including 11,342 treatment initiations	**NA[1]**

Summary of cohort ATUs

	2015	2016	2017	2018	2019
New cohort ATUs	13	10	11	20	**20**
Medicinal products under cohort ATUs having obtained an MA	12	9	8	16	**14**
Newly included patients	10,216	11,909	8,250	5,642	**3,766**

Total turnover of drugs under ATU in France in 2019 = 0,72 billion €

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Why EAP in France is complex?

- French authorities pioneered early access back in 1993 with dual process (compassionate use vs cohort use)
- Process is specific to France and not aligned with other EU countries pathways
- Daily management of ATU is time consuming, with numerous administrative steps
- Your drug must answer all ATU eligibility criteria below:
  - intended to treat, prevent or diagnose serious or rare diseases
  - no suitable treatment available on the market
  - efficacy and safety in use are presumed based on scientific knowledge and the start of the treatment cannot be deferred

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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 ATU

- **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 ATU 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 a detailed and standardized dossier to be prepared and submitted

  Submission and assessment process requires regular interactions with Authorities

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

PharmaBlue : your solution for EAP management

- PharmaBlue (a BlueReg company) is approved as « Exploitant » in France by French Regulatory Authorities (via a Wholesale Distribution Authorization - WDA)

- With this status, PharmaBlue is managing ATU since 2015:
  - We act as your local representative for the authorities
  - We endorse the full « Exploitant » responsibility for your ATU:
    - **Quality, Distribution, Medical Information, Pharmacovigilance, Communication**
  - We can provide support for distribution of your product in France if needed, through our preferred partner **Colca MS** (transportation, logistics and distribution)

PharmaBlue : your solution for EAP management

- Phase 1: We prepare ATU dossier, guide you through the submission
- Phase 2: Review process until approval
- Phase 3: We manage ATU 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

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Management of your ATU project

- **Projects Completed since 2015** *(as of May 2021)*

cATU completed

- **8 Projects**

Number of patients treated as part of A managed by PharmaBlue

- **Around 9600**

- **Projects in progress** *(as of May 2021)*

cATU currently active

- **4 projects**
- **250 patients**

cATU dossiers submitted, under evaluation by the ANSM

- **6 dossiers**

cATU dossier under preparation

- **2 dossiers**

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Get ready for the new Early Access regulation

New EA regulations to come into force from 1st July onwards*

- Existing scheme (namely ATU) will be replaced by «Early Access (EEA)» or «Compassionate Access (CAA)»
- Aim is to simplify the schemes that permit early access to new drugs to accelerate patient access
- EAA will be granted by the HTA body in France (HAS) and not by ANSM anymore
- Decision of the HAS will result in both the granting of early access and derogatory reimbursement by the health insurance system
- The idea is to have a "one-stop shop", allowing a simpler and faster procedure

* Article 78 of French Health System Finance Law for 2021 - Publication of the decrees & related orders are expected by the end of June 2021

Eligibiliy criteria updated

- Concerns the treatment of serious, rare or disabling diseases, when no appropriate treatment available
- Start of the treatment cannot be postponed
- Efficacy and safety of these drugs are strongly presumed from the results of therapeutic trials
- Drugs are presumed to be innovative, in comparison to a possible clinically relevant comparator

Definition of the “clinically relevant comparator” will be the key for early access eligibility

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Change in the rebate system for reimbursement

- Once EEA is granted, the company can continue to set a price which will be fully reimbursed
- Company finances the collection of data, ensures the continuity of treatment and commit to submit an MAA within a determined period
- New double mechanism of rebates:
  - annual rebates calculated on the turnover invoiced to French health system for the year concerned (considered as a provision mechanism for the 2nd type of rebates)
  - Rebates paid retrospectively at the exit of the scheme
  - Rates of these rebates subject to a progressive scale per turnover thresholds

Summary

What have we learnt 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
- “Exploitant” status is mandatory and PharmaBlue provide you with it
- PharmaBlue – Partner for your EA in France from start to end
- Support your Project to reach EU Market :
  - BlueReg – Partner of EU Coordination of Launch Activities & Promotional Material Review & LifeCycle Management

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Q & A Session

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Thank you

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