



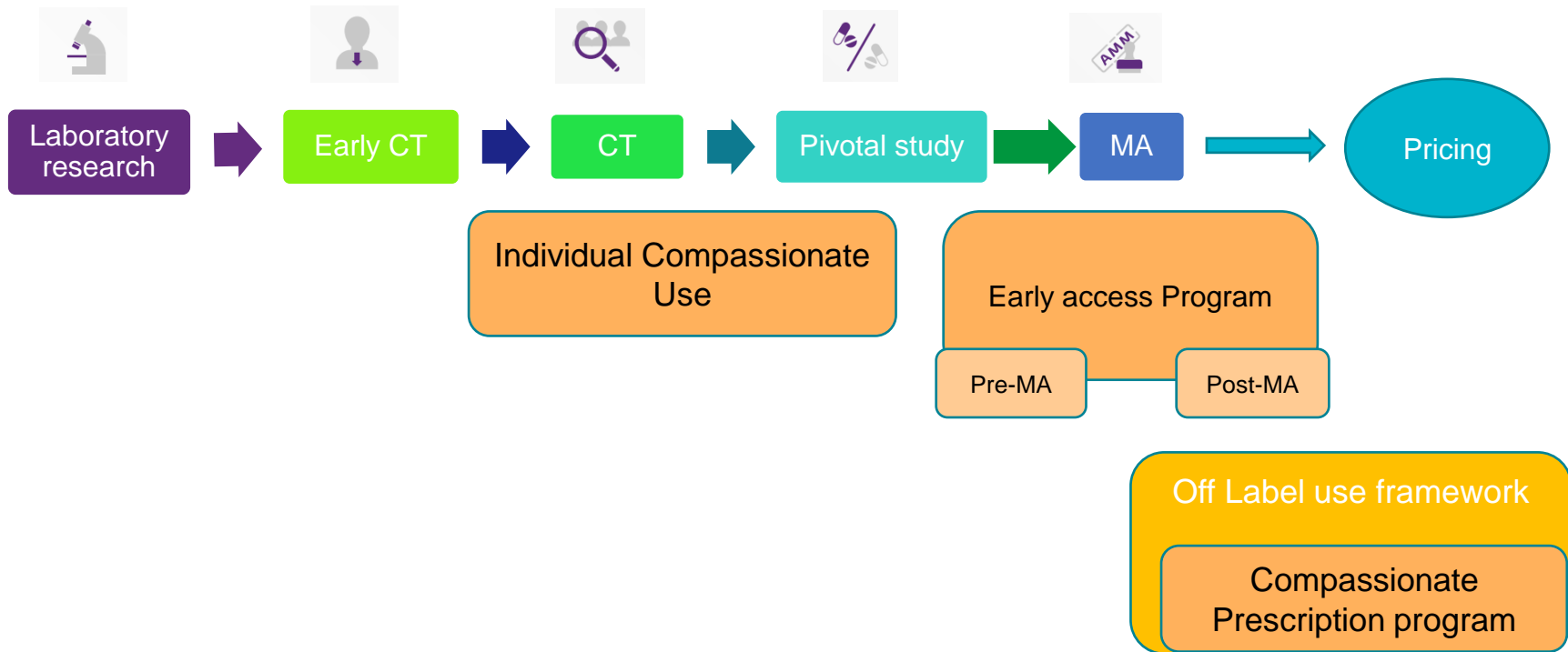
Agence nationale de sécurité du médicament  
et des produits de santé

# Accelerating access to treatments for patients

Dr Valérie DENUX  
Director Europe & Innovation

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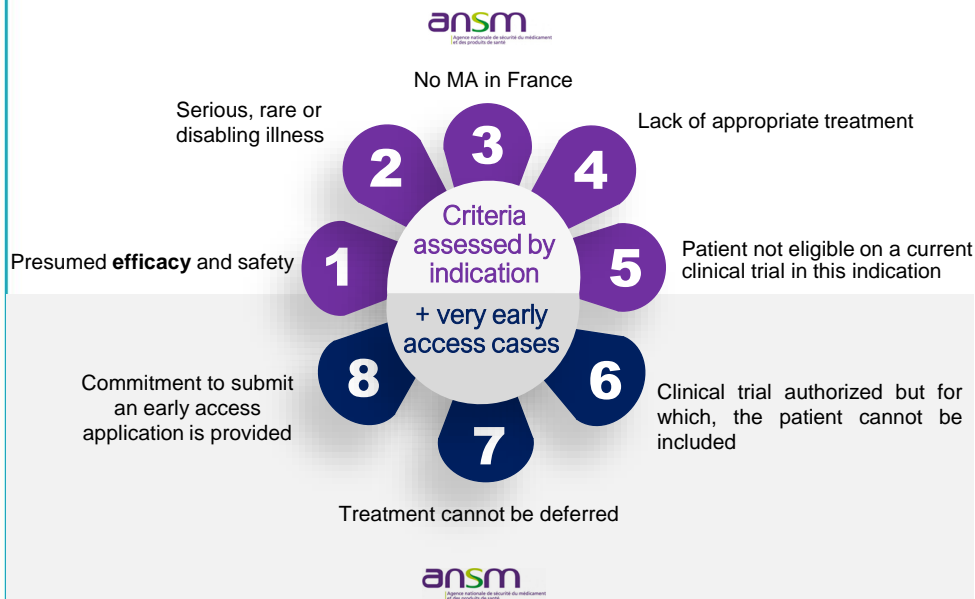
# Accelerating the access to Drugs through special authorizations



Special authorizations : new framework since July 2021

# Individual compassionate use (AAC) assessment on a case by case basis

## Assessment criteria



## Publication on the ANSM Website

- Definition of targeted population
- Protocol for therapeutic use
- Conditions of use
- Precautions for use and monitoring measures
- Summaries of periodic reports

Request by *Hospital* prescribers

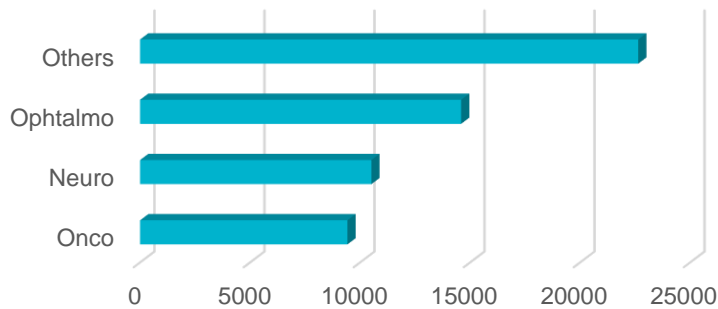
But GPs can refer patients to their hospital correspondent physician to ensure access to these innovative treatments.

Pharmaceutical companies agree or not to give access to their product through AAC

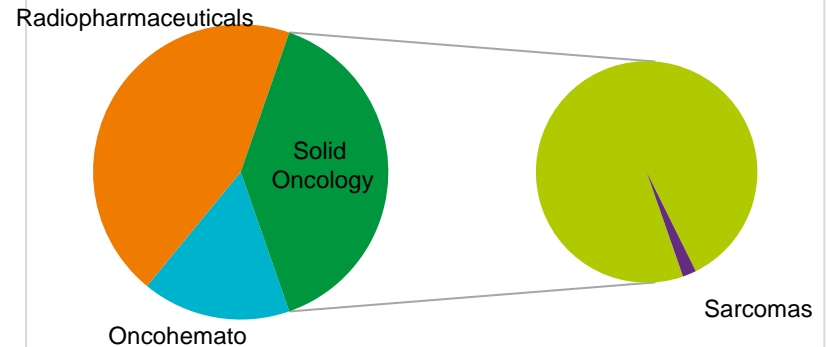
**Based on Directive 2001/83/CE – Article 5**

# Individual compassionate use in 2023 (AAC)

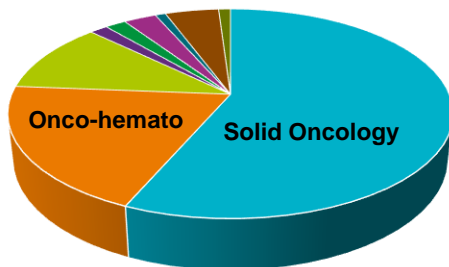
compassionate use authorization in 2023



oncology in the compassionate use program in 2023



Compassionate use authorization for drugs with a commitment to early access program



- Solid Oncology
- Hemato Oncology
- Neuro
- Rhumato
- Nephro
- Graft
- COVID
- Autre
- Pneumo

**12 drugs with commitments to AP are mainly used in oncology indications in 2023**

**More than 900 AAC have been delivered in oncology in 2023 for pediatric patients**

# Request Portal : E-Saturne

Créer une demande AAC

Demandes en brouillon 0 Demandes en attente de validation PUI 0 Je dois donner une réponse 0 J'attends une réponse de l'ANSM 0 Demandes en cours de validité 1

AAC à renouveler 0 AAC à renouveler (date de fin de validité dépassée) 1 AAC en cours ANSM 6 Toutes mes demandes 7

**Demande AAC** Total 0 Etat: Brouillon

Informations Générales			Médicament		Suivi	Décision		Patient		Prescripteur	
Numéro de dossier	Etat conversationnel	Type d'AAC	Solution thérapeutique	Date de transmission à l'ANSM	Date de la décision	Date de fin de validité	Nom Patient	Prénom Patient	Date de naissance Patient	Nom Prescripteur	Prénom Prescripteur

- ◆ Secured and extended access through a e-CPS connection
- ◆ Easy search (brand name, INN, indication, etc.)
- ◆ Communication via Programmable alerts and notifications
- ◆ Rapid identification of actions to be taken (banner system)
- ◆ Easier for piloting authorizations

# Early access authorization (AAP) patient cohort evaluation

*Based on Regulation 726/2004 – Article 83*



Treatment cannot be postponed



Serious, rare or disabling illness



Lack of appropriate treatment



Positive benefice/risk for patients



Presumed to be innovative, particularly with regard to a clinically relevant comparator

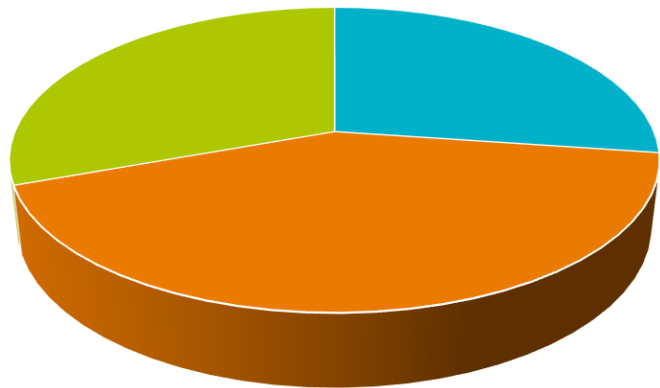
Hospital prescribers and general practitioners (particularly in certain health situations - COVID) can include their patients in these cohorts, provided they meet the conditions described in the Protocol for Therapeutic Use (PTU)

***More than 100 000 patients treated in this  
framework since 1st of July 2021***

# Early access authorization

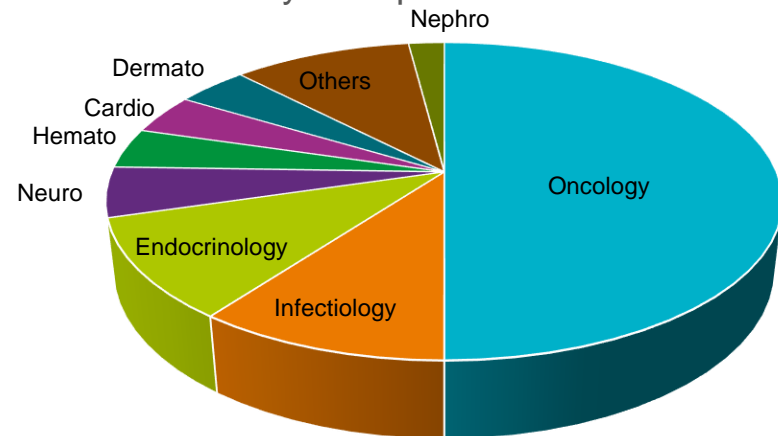
180 decisions over the past 2 years, including 125 new products

AAP decision since July 2021

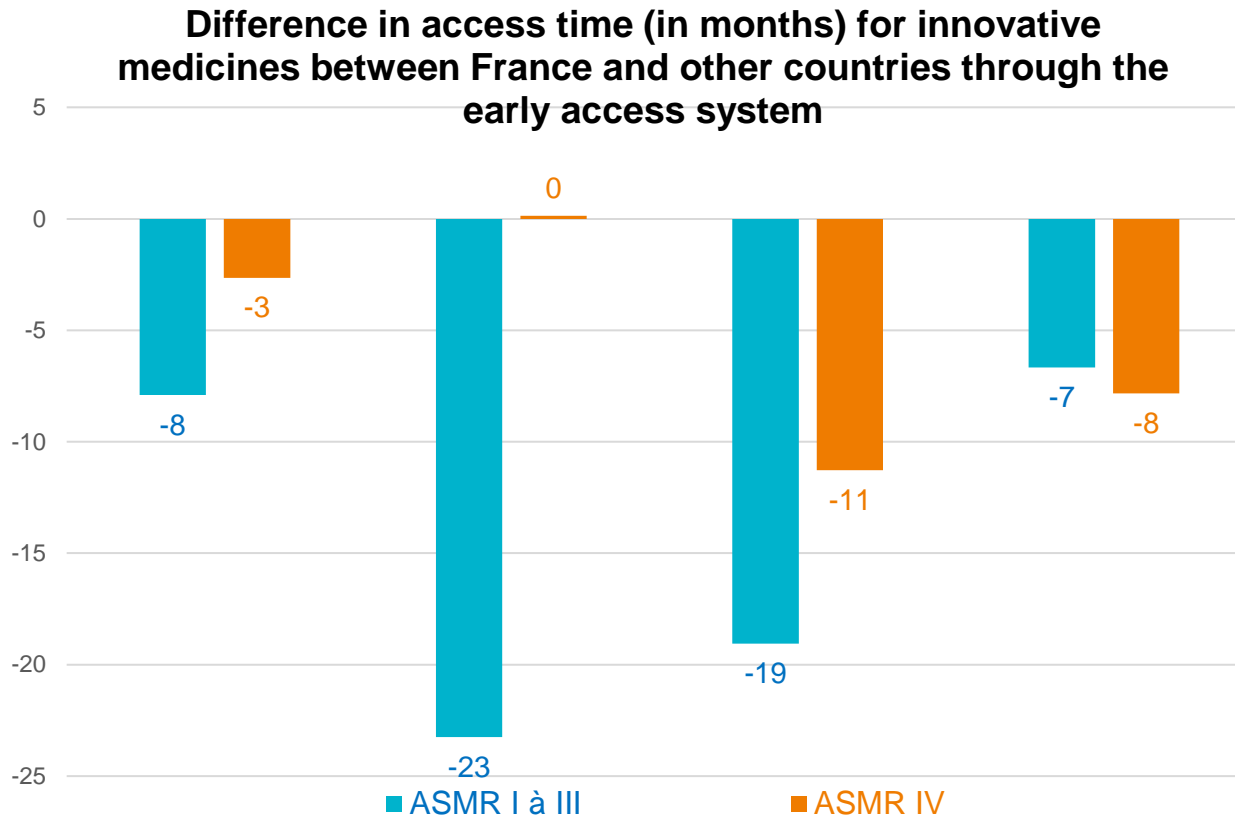


■ pre-MA Program ■ Post-MA Program ■ AP Prolongation

Favorable decision for early access program by therapeutic domain



# Special authorizations: accelerating the access to treatments

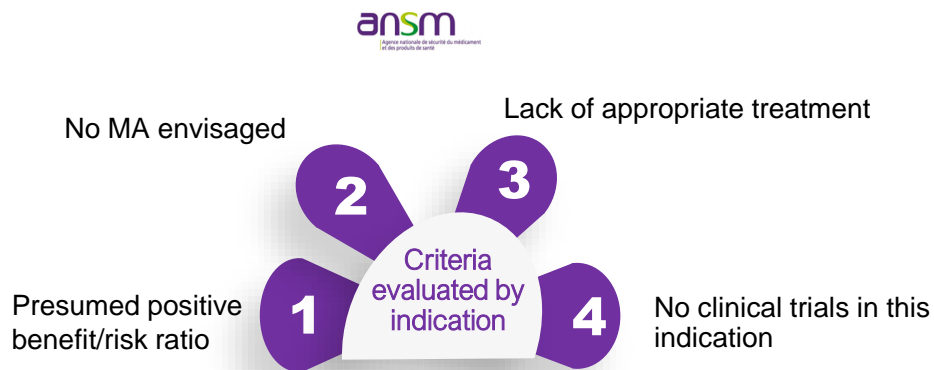


On average, patients have access 11 months earlier to drugs through the early access system with clinical benefit before a price is published in one of the corridor countries.



# Compassionate Prescription program (CPC) - off-label use

## Assessment criteria



## Accelerate access to new indications

***Based on Directive 2001/83/CE – Article 5***

## Publication on the ANSM Website

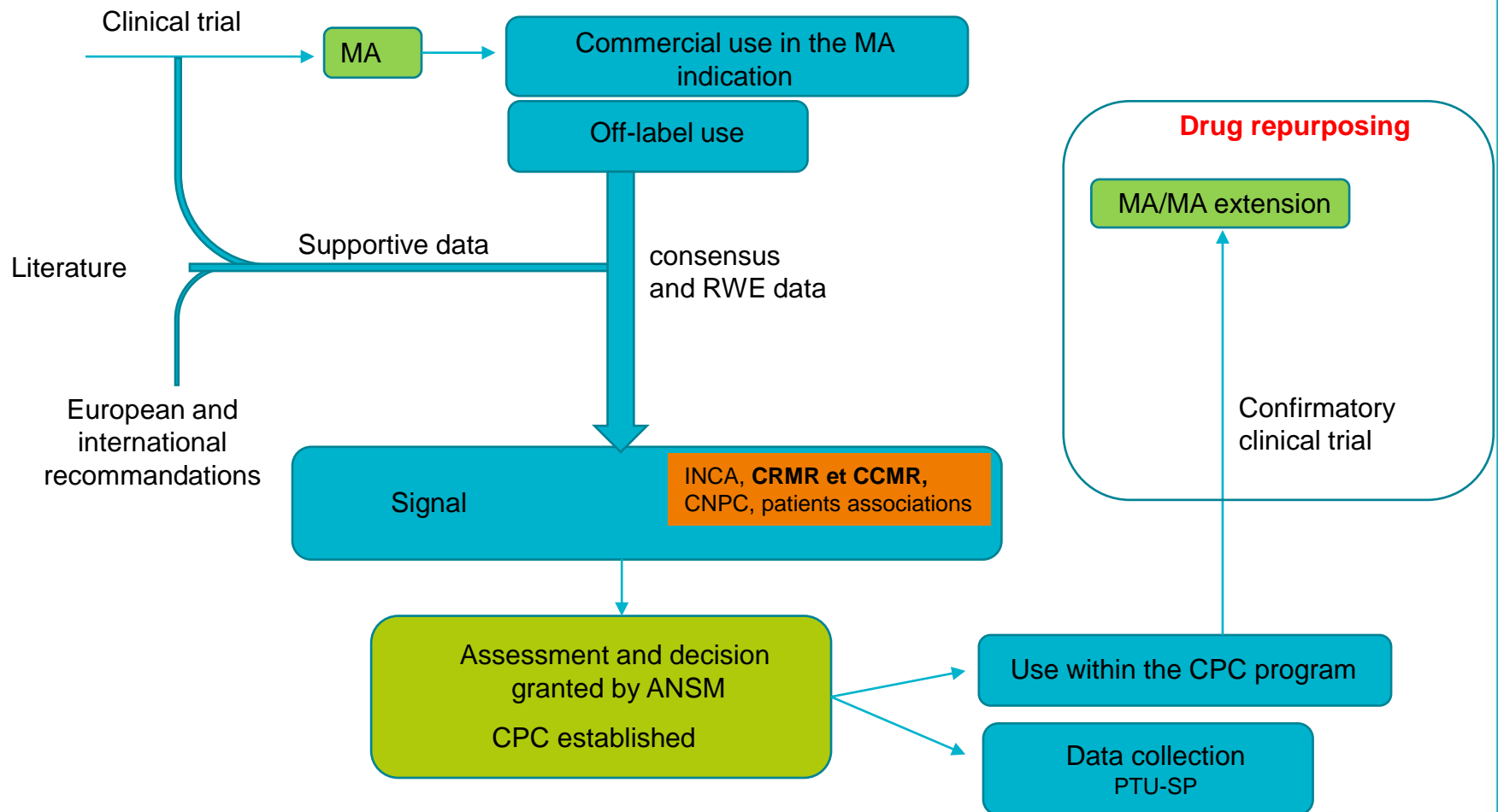
- The decision
- The protocol for therapeutic use (PTU)

Implies off-label use to be reported by healthcare professionals, patient associations or institutions

Hospital prescribers and general practitioners can prescribe these CPC-eligible treatments (outside the AMM), provided they meet the conditions described in the PTU.

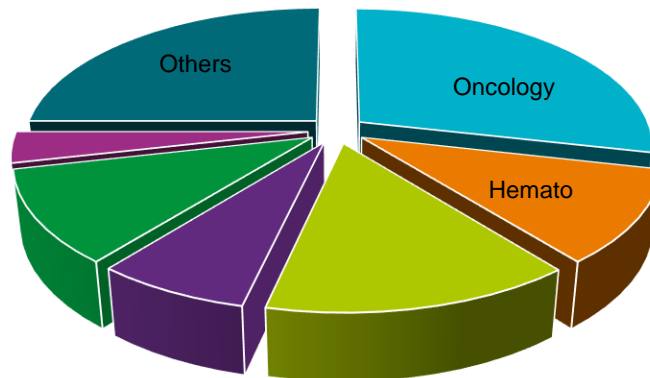
Provided reliable data collection can help for designing confirmatory clinical trial through repurposing process

# Compassionate Prescription program (CPC)



# Compassionate prescription program off-label use (CPC)

Authorized CPC by therapeutic domain



■ Oncology ■ Hemato ■ gyneco ■ endocrino ■ autoimmun ■ Neuro ■ others

- 28 CPC currently authorized, half of which in rare diseases
- 14 signals within the instruction process
- 91 signals of interest in rare diseases prioritised and to be signaled following off-label use identification

# Conclusions

- ◆ **Thousands of patients accessing treatments through special authorization programs**
- ◆ **Patients access to innovative treatment acceleration**
- ◆ **Difficulties related to data collection (time consuming, heterogeneity, etc)**
- ◆ **Need to strengthen data analysis and utilization**

The lower half of the slide features a teal background with a white diamond shape. The diamond is tilted and partially overlaps a white line that forms a jagged, mountain-like silhouette. The text is positioned on the right side of the teal area.

### Avertissement

- Lien d'intérêt : personnel salarié de l'ANSM (opérateur de l'État).
- La présente intervention s'inscrit dans un strict respect d'indépendance et d'impartialité de l'ANSM vis à vis des autres intervenants.
- Toute utilisation du matériel présenté, doit être soumise à l'approbation préalable de l'ANSM.

### Warning

- Link of interest: employee of ANSM (State operator).
- This speech is made under strict compliance with the independence and impartiality of ANSM as regards other speakers.
- Any further use of this material must be submitted to ANSM prior approval.



**60' Lunch break**

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**THE SESSION WILL START AT 13.30 PM CET  
or 09.30 am EST time**

