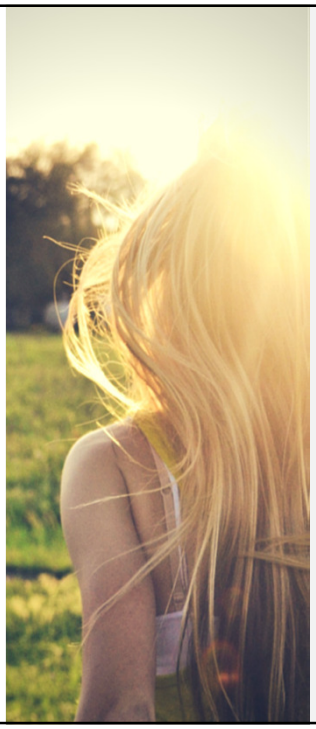

Dutch association
Innovative
Medicines



# Future topics for regulatory science

## An innovators perspective

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Association Innovative Medicines

Lygature Partnerships MeetUp
12 October 2017, Utrecht

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## Goal

To help more patients earlier that need cutting edge treatments

through acceleration of early access to innovative medicines for appropriate patient groups

whilst maintaining safety and efficacy standards

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**The patient** does not exist

**The medicine** for an entire population does not exist

Due to scientific progress and rapidly increasing innovative treatment and diagnostic options **patient populations become smaller but better defined**

Many **unmet medical needs**

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## Current possibilities @ EMA

Targeting unmet medical needs or major public health interests

- Supporting development process from early stage
- Offering regulatory mechanisms to help promising new medicines reach patients as early as possible
- Included in EU legislation:
  - accelerated assessment
  - conditional marketing authorisation
  - compassionate use
- EMA PRIME (PRIority MEdicines) scheme

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## Under development in EU

1. Adaptive Pathways @ EMA

- **Scientific concept** for **medicine development** and **data generation** which allows for **early and progressive patient access** to a medicine
- Using **existing** European Union (EU) **regulatory framework** for medicines

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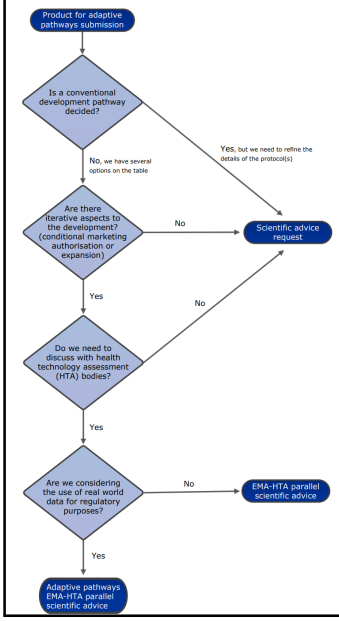


## Adaptive Pathways @ EMA

Three principles

- **iterative** development
  - approval in stages, from restricted to wider patient populations
  - confirming benefit-risk balance after conditional approval based on early data (using surrogate endpoints)
- **gathering evidence through real-life use** to supplement clinical trial data
- **early involvement** of **patients** and **health-technology-assessment bodies** in discussions on a medicine's development

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HORIZON 2020

efpia
European Federation of Pharmaceutical Industries and Associations

## Under development in EU

2. Medicines Adaptive Pathways to Patients (MAPPs) @ IMI

- prospectively planned, iterative approach to medicines development and access pathways
- within current regulatory framework
- optimises early patient access, public health and societal benefits

ADAPT SMART project ADAPTSMART

- novel multi-stakeholder enabling platform how to put MAPPs into practice in Europe

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## What is needed

- (International) **Multi-stakeholder dialogue and cooperation,** no single party can do this alone
  - patients, regulators, HTA/payers, practitioners, and innovative companies
- **Integrated** end-to-end approach to **evidence generation**
- Decision making **across** the medicines **lifecycle**
- Based on **(regulatory) science**
- Discussion on acceptibility by stakeholders of
  - **Real World Evidence & Data** (RWE & RWD)
  - Other **novel sources of data** (e.g. continued evidence generation, historic controls, indirect comparisons, …)

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## Destination more important than route

Which regulatory **route** or process to use and what evidence to generate should always be **supportive** to our **goal**: timely and prospectively planned patient access to innovative therapies

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