European perspective: Results of survey on early access schemes / compassionate use of new medicines

Flow of the Presentation

- What is compassionate use? When is it used?
- What was the “survey” and why did we do it?
- What were the findings of the survey?
- Discussions with regulators

What do we mean by early access & compassionate use mechanisms?

- Early Access
  - Mechanisms that can shorten the time it takes to get from bench to full market access for patients
  - PRIME, accelerated assessment, conditional approval, exceptional circumstances, MAPPS & adaptive models, joint scientific advice with payers
  - Can also be used to describe “compassionate use” schemes
- Compassionate Use
  - Provision of a drug to patient(s) outside of a clinical trial and before a product is licenced (and available) in a market
  - The presentation will focus on compassionate use – other speakers have covered “Early Access mechanisms”

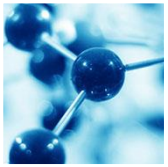
Overview of EU Systems for Compassionate Use (& a little bit of CTs)

Individual Patient Access

- Based on article 5 of 2001/83/EC
- Request from patient or Dr to manufacturer
- National MS legislation

Cohort/group Patient Access

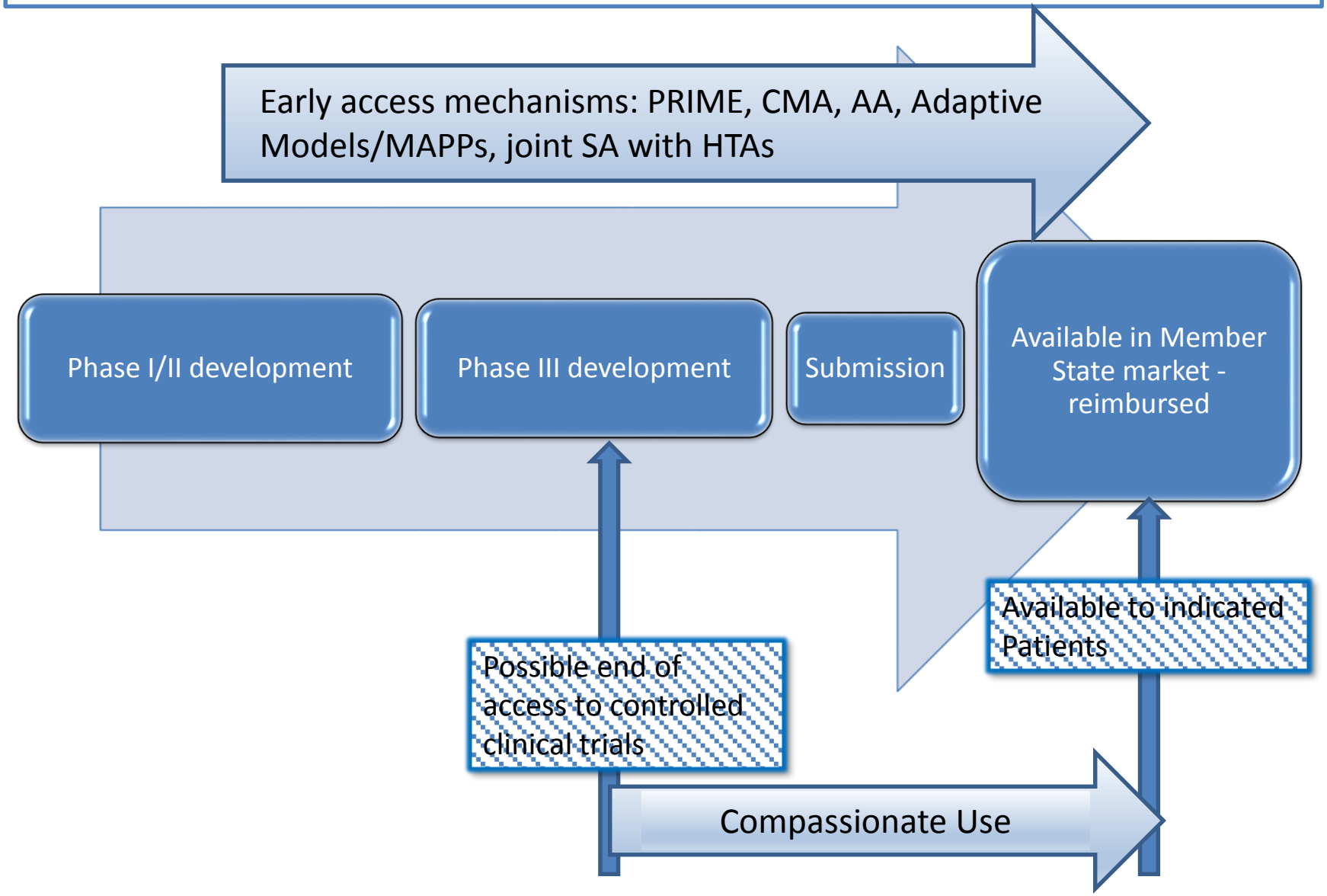
- Article 83 Regulation 726/2004
- Member states request CHMP opinion on use for a cohort of patients



Early Access Clinical Study

- Clinical trial with primarily a safety endpoint (incl. extension studies)
- Supply to eligible patients at specific sites only

Typical Timing for Compassionate Use



What was the survey? Why did we do it?

- We asked organisations to send us examples of their experience of CUP mechanisms, any issues they encountered and their proposals for how these may be resolved in the future
- The focus was on patient access and we wanted to learn about recent experiences, hurdles, concerns and was there scope to include compassionate use as an area for review by STAMP*
- The outcome was remarkably similar feedback from several companies

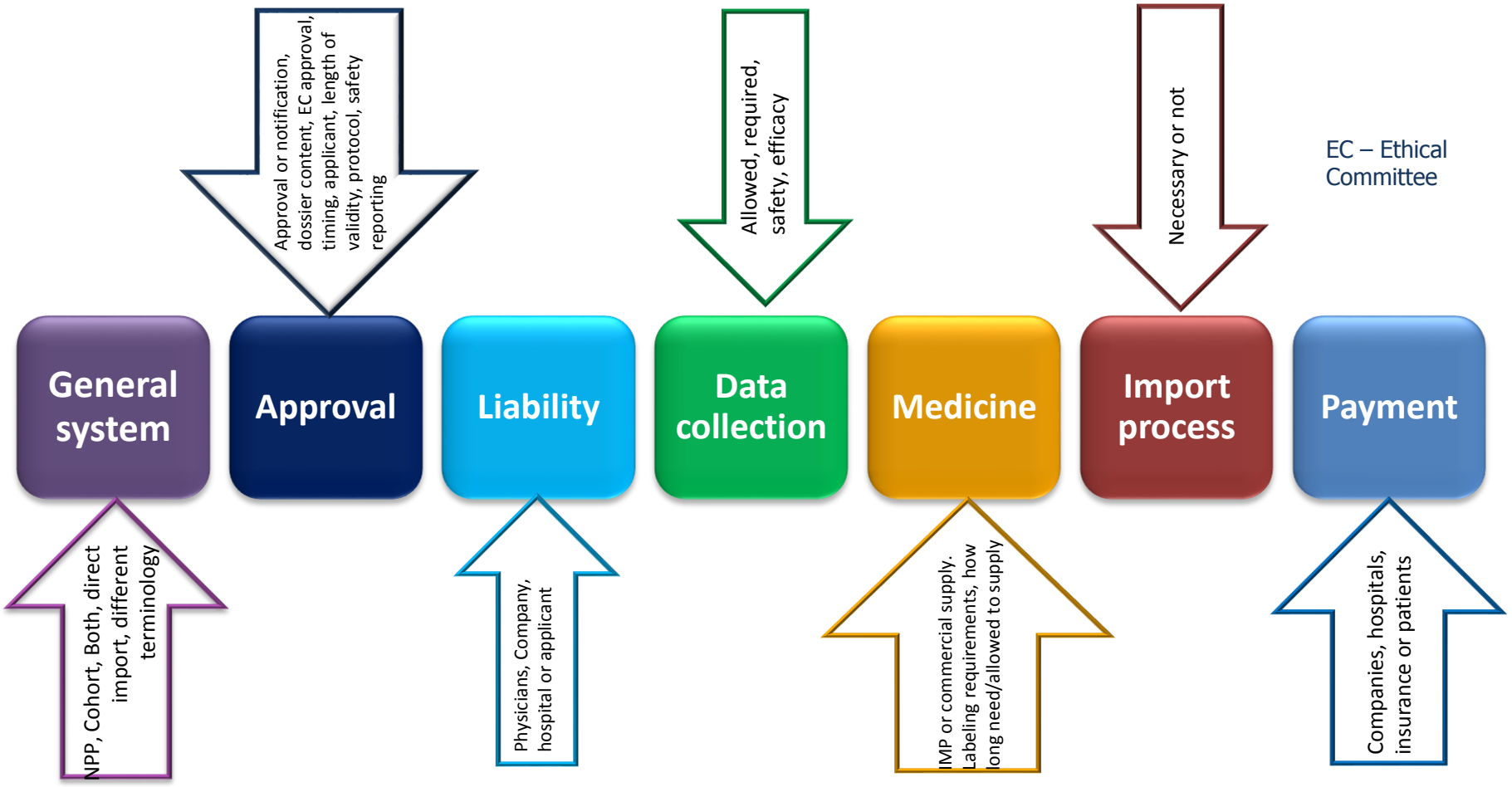
*STAMP - Commission Expert Group on Safe and Timely Access to Medicines for Patients

The STAMP will identify ways to use more effectively the existing EU regulatory tools with the aim to further improve safe and timely access and availability of medicines for patients.

Outcome of the survey - summary

Specific Differences in the Countries' Schemes

Thanks to EFPIA and BMS for this slide



What were the proposals discussed with STAMP?

Priorities for Improvements (1)

- **Enhancing Integration of Patient Perspectives**
  - Need for education/ information of patients of early access programs via patient groups
  - IMI projects may explore/identify opportunities and methodology to enhance patient perspectives and understand when/how these could be used.
  - Need for an equal chance for patients to early access medicines across MSs:
    - Different approaches by Member States on compassionate use lead to disparities in access to new innovative medicines by patients
    - Alignment is required between different national compassionate use systems in particular with respect to scientific criteria and procedures
    - This would improve equitable access and provide a decreased administrative burden and procedure time for early access

Priorities for Improvements (2)

- **Apply schemes to new indications of authorised meds**
  - Would benefit from controlled compassionate use mechanisms
  - Current practice for access is via “off-label” use
  - *(Of note – need to assess legal feasibility under existing legislation)*
- **Stronger alignment of Member States**
  - Different approaches by MS on compassionate use lead to disparities in access to new innovative medicines by patients
  - Alignment is required between different national compassionate use systems in particular with respect to scientific criteria and procedures.
  - Establishment of a framework for cohort CUPs in each MS for Article 83 to be fully operational is critical

Priorities for Improvements (3)

- Enhance use of Art. 83
  - From the available Art. 83 CU Opinions it seems that very few MSs have requested it (Ireland (1), Sweden (3) and Finland (1))
  - Research (survey/ study by COM/EMA) needed on
    - Root cause analysis why MS are not requesting an Article 83 opinions
    - Root cause why only few MS established the framework to support cohort CUPs
    - Overview on ongoing CU Programs in MS (through MS information to EMA) and potential need for improvement for reporting mechanisms to EMA

Priorities for Improvements (4)
Article 83 Process

- **Request**
  - Currently only MS can request Art. 83 – need to consider how Patient Groups and Industry can trigger requests (via MS)
- **Connection with other tools (data driven):**
  - PRIME designation or use of Adaptive Pathways could trigger the rapporteur MS to request an Article 83 opinion after consultation with the applicant. This would potentially improve take up of “centralised” compassionate use opinions
  - Utilise CU Programmes to allow for critical real world data gathering
- **Procedure & timelines**
  - Further clarification on requirements and timelines to be detailed and published by EMA (also as part of CHMP guidelines)

Discussions with Regulators

- STAMP has discussed CUP and the national level challenges already in 2015. Learnings to date were discussed with CHMP in Jan 2016, with the aim on sharing views on the topic and identifying how the framework could be better utilized
- There has been a recent discussion between EFPIA and the STAMP team which included member state representatives. Some of the feedback from the regulators was interesting and critical to bear in mind
    - There was concern that Compassionate Use was misused and openly discussed with physicians, inviting them to participate – clearly this is not acceptable. The legal basis is "*a bona fide unsolicited request*"
    - Reimbursement approach by Industry was unknown – this requires follow up by the EFPIA team
    - Terminology such as "programme" and "real-world data" needed clarification

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