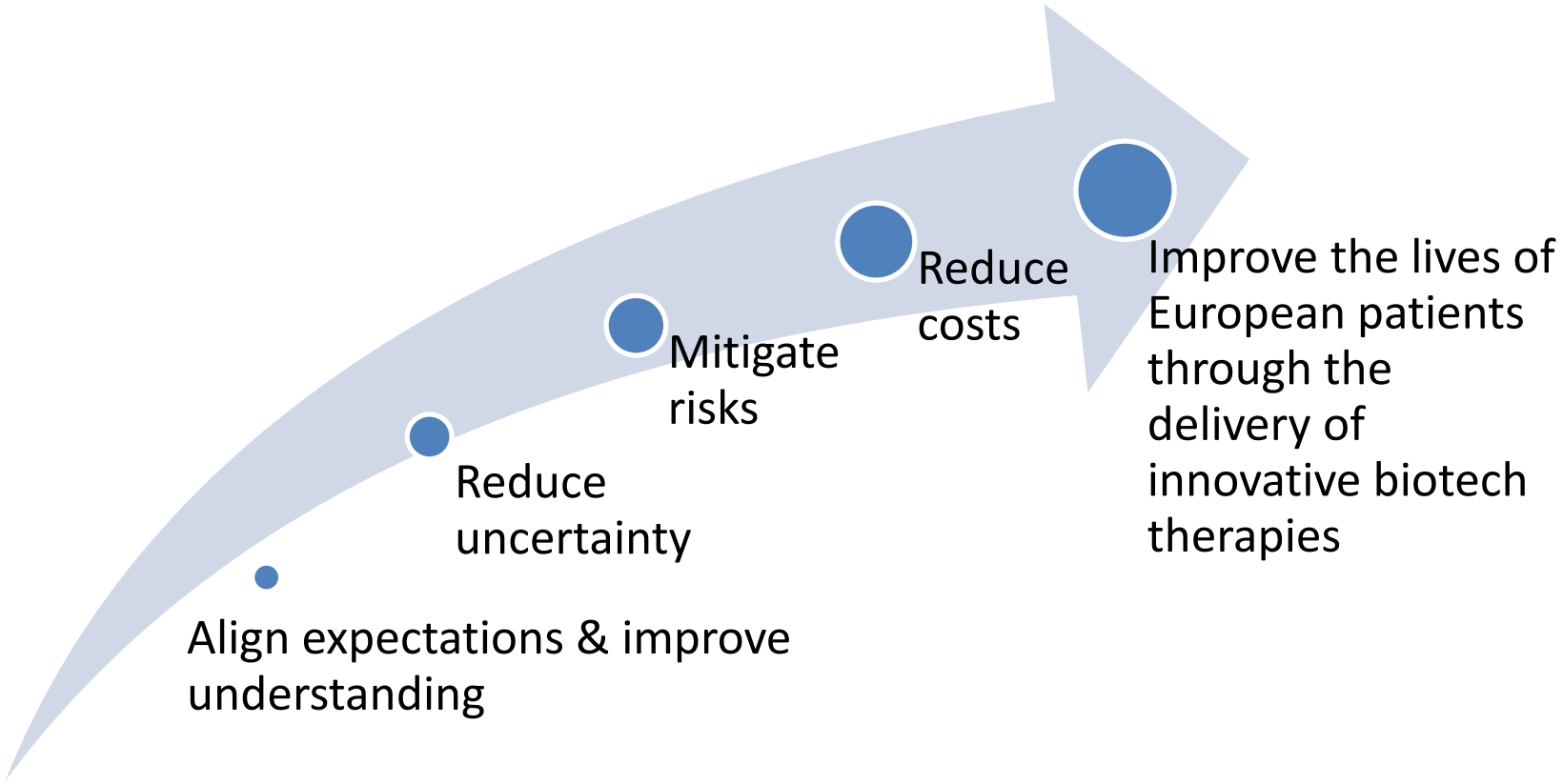
EuropaBio™

The European Association for Bioindustries

EMA and HTA Advice in Europe: Experience of Biotech SMEs

EuropaBio **20/11/2013**

HTA advice activities, a major opportunity for EU healthcare stakeholders

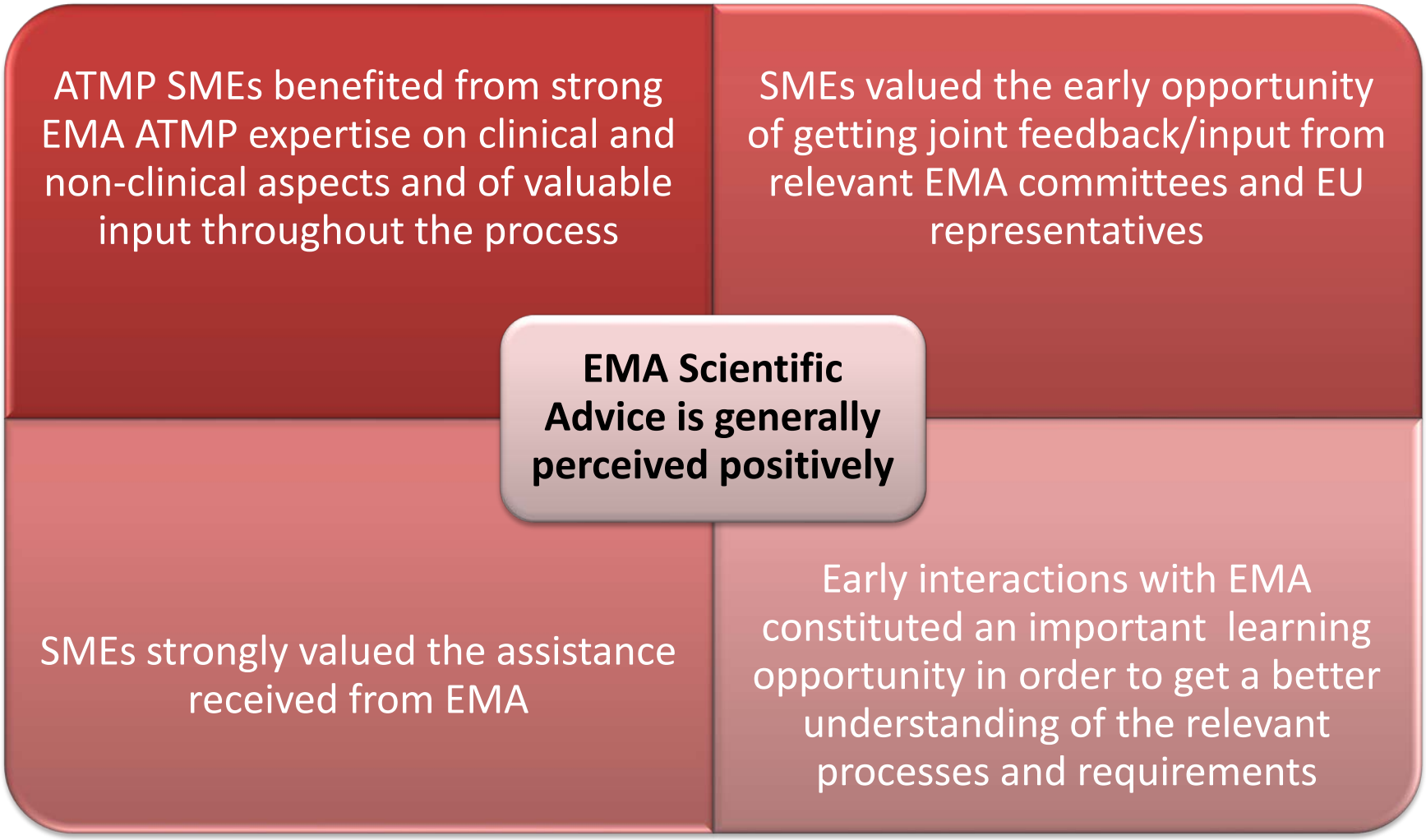


EuropaBio 2013 Survey: preliminary outcomes

- The EuropaBio survey has the objective to outline the perceived benefits, challenges and opportunities for SMEs when engaging in EMA and HTA advice activities
- The survey is being conducted through phone interviews with SMEs, EMA and a number of HTA bodies
- Today's presentation will focus on the outcomes of the interviewed SMEs only

Interviewed SMEs

- 8 SMEs participated in the interviews, 4 developing ATMPs, 5 developing OMPs
- All companies have manufacturing activities in Europe, 7 out of 8 also have their discovery and R&D activities in Europe
- Collectively they have accumulated 15 phIII and 7 phII ongoing trials
- Only 1 company has a dedicated HTA FTE, some companies outsource a maximum of 2 FTEs
- On average, they employ 143 employees of which 3.5 FTEs have regulatory expertise and 80 FTEs are in R&D functions


ATMP SMEs benefited from strong EMA ATMP expertise on clinical and non-clinical aspects and of valuable input throughout the process
SMEs valued the early opportunity of getting joint feedback/input from relevant EMA committees and EU representatives
EMA Scientific Advice is generally perceived positively
SMEs strongly valued the assistance received from EMA
Early interactions with EMA constituted an important learning opportunity in order to get a better understanding of the relevant processes and requirements

But the interviewed SMEs also encountered a few challenges

1) **Process:** For some, the EMA process was too lengthy and cumbersome, lacking the flexibility that SMEs require
2) **Input:** Sometimes input was considered to be vague or inconsistent over time
3) **Investment/capability:** Engaging in the EMA's Scientific Advice process requires a significant effort for an SME – this effort may not always be sufficient to reach the expected outcome

The majority of interviewed SMEs did not engage in early HTA advice activities

1) **Lack of awareness:** They were not aware of it or didn't have enough expertise

2) **Timing:** They thought to be too early in the development phase; they have out-licensed commercialization (and HTA activities with it), or they were fully focused on the regulatory process

However, they are willing to do it in the future…

1) Because early HTA advice can help them better understand HTA requirements and add new, relevant information in the choice of comparator(s) and/or endpoints

2) More generally, it can provide a learning opportunity and also a way to improve HTA bodies' understanding of rare diseases and of new therapeutic approaches

The interviewed SMEs would further engage in HTA advice if some structural constraints could be partially removed

1) **SMEs' lack of awareness, expertise and resources**: the lack of HTA expertise and resources within the interviewed biotech SMEs – also among R&D and regulatory employees – makes them unaware of the necessity of engaging in early HTA advice activities

2) **Highly complex HTA environment:** the multiplicity of HTA bodies, at regional and/or national level, and the diversity of approaches and requirements by each HTA body does not pave the way for effective interactions between HTA bodies and SMEs. SMEs fear the costs associated with engaging in early advice discussions and they also lack the resources to address different or inconsistent inputs into their clinical plans.

Concluding Remarks

EuropaBio and SMEs value regulatory and HTA early dialogue and advice activities as a way to address uncertainties during the drug development phase

Biotech SMEs face major structural challenges in engaging in HTA advice, these are related to their own limited resources and expertise but also to the complexity arising from a multitude of HTA requirements across Europe

EuropaBio wishes to engage with the EU Commission, the EMA, the national HTA agencies, EUnetHTA and the European HTA Network in identifying , designing and implementing practical solutions to the above mentioned challenges

In particular, SMEs have identified EMA/HTA's joint training and advice activities as the ones that can best address current challenges

Thank You

For further information please contact:
Paolo Morgese
Chair of the EuropaBio HTA Topic Group
Director of Research at Deerfield Institute
pmorgese@deerfield.com