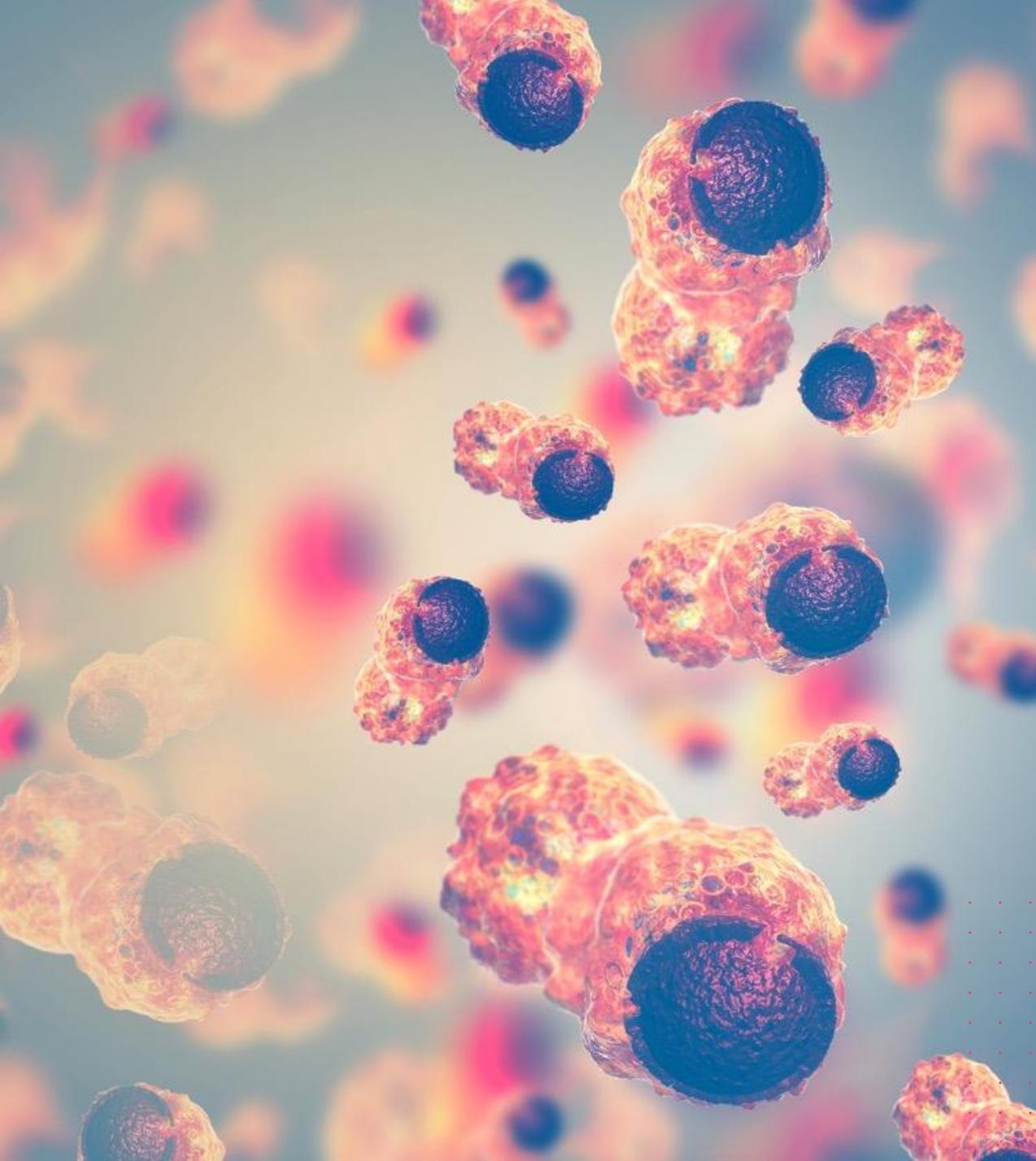


Swelife ATMP SDP3 - Business models and health economics



A microscopic view of various cells, likely cancer cells, showing their irregular shapes and dark nuclei. The cells are scattered across the frame, with some in sharp focus and others blurred in the background. The colors range from bright yellow and orange to deep red and blue.

Swelife ATMP project on Health Economics and Business Models

AIM:

- > Map the landscape**
- > Address barriers around valuation, payment models and affordability of ATMPs**

Long term goal:

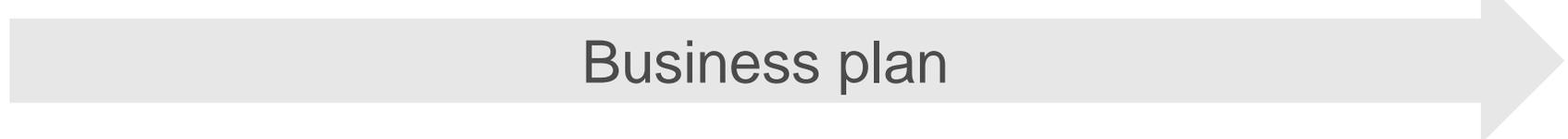
- > To promote successful development and implementation of ATMPs in Sweden.**

Project partners	Persons involved
Akademiska Sjukhuset	Alexandra Karlström, Anna Björkland
CellProtect Nordic Pharmaceuticals	Karin Mellström
Idogen	Agneta Edberg
Institutet för hälsoekonomi (IHE)	Ulf Persson, Peter Lindgren
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Region Västerbotten	Örjan Norberg, Elham Pourazar
RISE (Research institutes of Sweden)	Anna Ridderstad Wollberg (project leader), Ronja Widenbring, Charlotte Nilsson
Swelife	Ebba Carbonnier (Styrgrupp)

November 2018



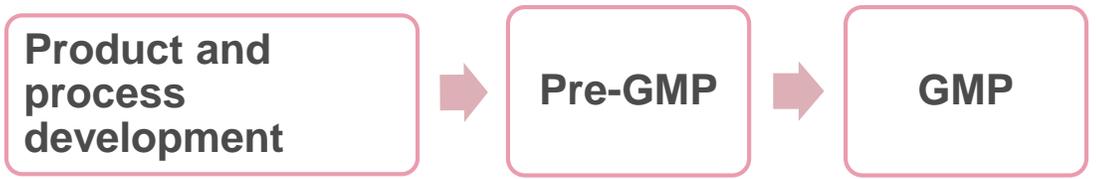
ATMP Development path

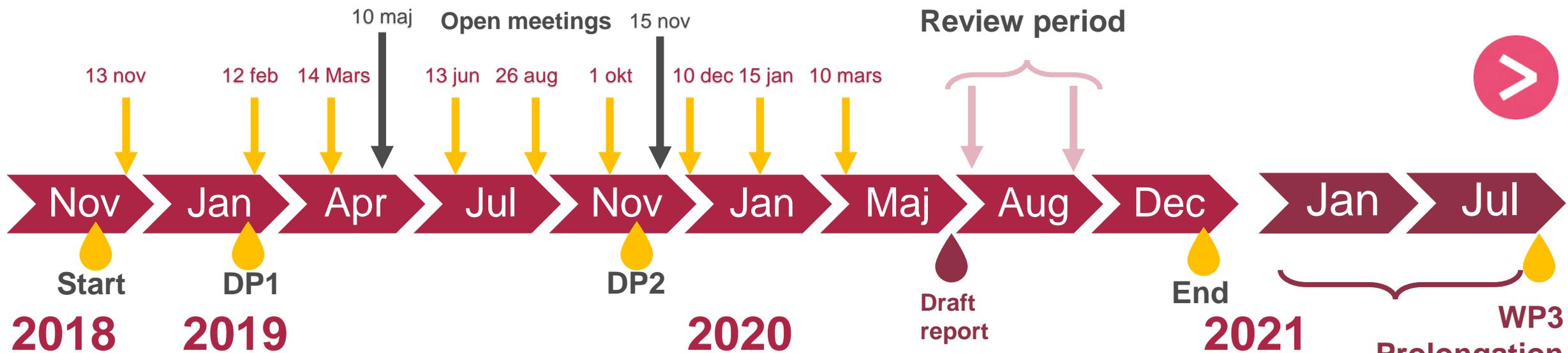


Disease knowledge Screening, selection optimisation IMPD IB **Reimbursement** Follow up Real world evidence



Challenge





Definition



Mapping
Stakeholders
Roadmap



- TPP cases
- HTA in other countries

Stakeholder interactions



HTA and payment models

- Report
- Checklist – guide
- Roadmap
- TPP cases

WP3
Prolongation
Sub-team
Örjan Norberg
Reg Västerbotten

Innehåll

Slutrapport för Swelife-ATMP systemutvecklingsprojekt 3 (SDP3)

Förkortningar

Sammanfattning

 Projektets huvudbudskap

Bakgrund och metoder

 Team och projektparter

Introduktion

 Projektets avgränsningar

 Projektets målgrupper

 Effektmål

Resultat från projektarbetet

 Problembeskrivning

 Värdet av en behandling

 Roadmap

 Legala aspekter..... **Roadmap**

 Sjukhusundantaget

 Generiska målprofiler (TPP) för typfall av ATMP

 HTA och hälsoekonomiska utvärderingar i Sverige

 Beskrivning av HTA.....

 Komparatorn

 Etiska plattformen..... **HTA**

 TLV..... **Mapping**

 Folkhälsomyndigheten.....

 SBU

 Socialstyrelsen

 Regionernas samverkansmodell

Mer om HTA och läkemedelsinförandet i Sverige..... 25

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 Stor "cost-offset"..... 33

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Betalningsmodeller..... **Payment models**

 Icke-resultatbaserade betalningsmodeller..... 38

 Resultatbaserade betalningsmodeller..... 38

 Innovativa betalningsarrangemang kopplat till ATMPs egenskaper

 Utveckling av innovativa resultatbaserade betalningsmodeller vid ATMP

Översikt av HTA-processen i andra länder

 Övergripande sammanfattning om hälsoekonomisk analys av globala marknader

 Hälsoekonomisk analys av ett urv..... **HTA practice in other countries**

 Hälsoekonomisk analys av de asi.....

 Hälsoekonomisk analys av den n.....

Web-sökningar om ATMP och hälsoekonomi.....

Referenser

Bilagor

A. HTA Checklista

B. Vägledning HTA Checklista

C. Generiska målprofiler (TPP) för ATMP

D. Värdebaserad prissättning

E. Regelverk

F. Den genetiska sjukdomen FAT-TTR, Skelleftesjukan

G. PPT slide deck HTA Europe, Asia

H. Rapport ATMP Market overview Asia (ADB)

I. Rapport ATMP Japan Sakigake (ADB)

J. Rapport ATMP_HTA mapping Europe (monocl)

Appendices



Target groups for the report



> The report mainly focuses on the novice developer perspective

> Start-ups / SMEs

➤ Usable tools

> Mapping HTA landscape (ATMP focus)

> HTA Checklist

> Roadmap

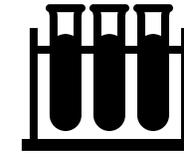
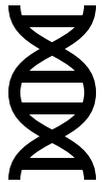
> Generic cases

> Formal/informal tollgates

> HTA practice (Sweden and abroad)

> Payment models considerations

> The report will also be useful for incubators/innovation offices and big pharma for example



Stake holders in the ATMP landscape

> Patients and organisations

> Academic groups, researchers

> Innovation support system

> Start-ups, SMEs

> Big Pharma

> Health care system personnel

> Payers (venture capital, research funding)

> Politicians

> Authorities (TLV, LV)

> Regions, SKR



Generic Target Product Profiles for ATMPs



- > **A summary of the target profile of the final product**
 - > What is the product, how will it be used, patient need...
- > **What do the project need to deliver for the product to succeed?**
- > **Efficacy target @time for market introduction**
- > **What is needed to pass the following “toll gates”**
 - > Regulatory approval
 - > Health economy
 - > Willingness to pay
- > **A systematic table covering these different aspects**



Fictive cases

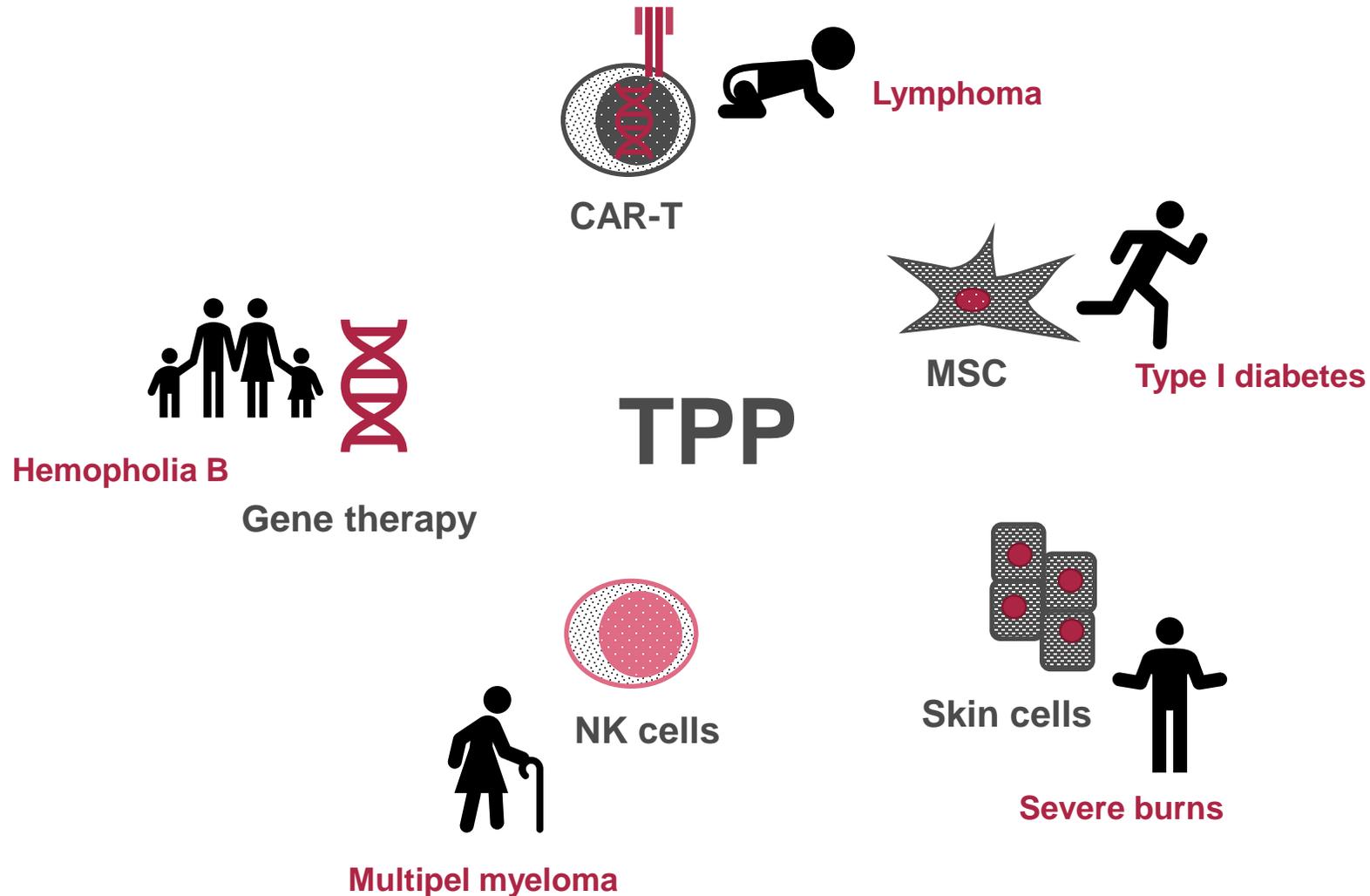
Generic Target product profiles for five different ATMP cases

Ambition is “fit for purpose”

Aim is to inspire understanding of “what do I need to think of for my own project



ATMP's come in many different forms



Project approach to TPP

- Patient perspective
- Product profile
- Payor perspective
- Product developer / investor perspective
- Societal perspective

Project approach to Target Product Profiles (TPP)

Case #
Type of ATMP
"One liner"
Descriptive case definition
General profile
General description
Intended indications and use
Target subpopulation as defined by inclusion and exclusion criteria
Biomarker or other specific diagnostic for precision medicine
Medical need / disease severity
Posology (dosing and route of administration)
Cell source - patient's own, other patient or other
Mechanism of action
Clinical efficacy - primary endpoint in pivotal trial
Definition of patient population basis for regulatory approval
Safety
Other requirements
Product characteristics - dosage form, shelf life, storage and transportation conditions
Requirements for qualification of treating health care unit (if applicable)

Payor perspectives
Epidemiology - incidence, prevalence, subgroup breakdown
Relative effectiveness (how intervention is targeted to compare to current standard of care)
Health economics, e.g. incremental cost effectiveness ratio (cost per QALY), net budget impact
Existing or presumed health economic uncertainties, e.g. imprecise target patient group definition, lack of direct comparator (only indirect, modelled), high degree of uncertainties in assumptions, lack of long-term follow-up
Price reference comparator
Patient perspectives
Unmet need
Anticipated gain
Anticipated risk or burden
Product developer/investor perspectives
Development stage
Remaining development activities
Remaining cost of clinical development
Remaining cost for manufacturing scale up and CMC dossier development
Anticipated manufacturing failure rate or other cost drivers
Pricing comparator
Cost of goods sold (rough estimate)



What is Health Technology Assessment, HTA?

> A systematic evaluation of "methods" in health care

From a **> Medical**
> Economical perspective
> Ethical
> Societal

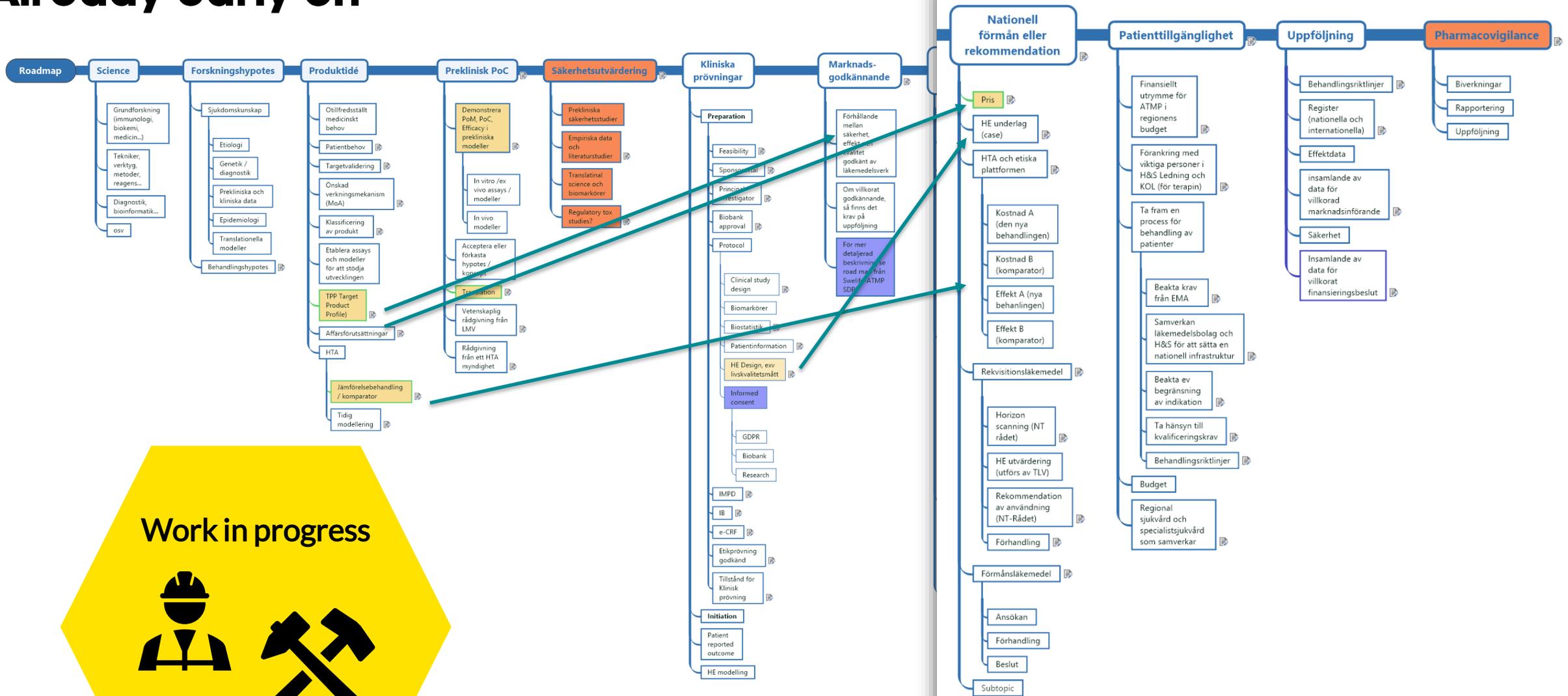


> Recommendation?

> Reimbursement?

ATMP Roadmap Keep the end-goal in your mind

Already early on



Work in progress





Main take home

- > **A well planned development path, based on formal mile stones**

 - Reduce economic and product related risks and increase project value

- > **In developing ATMP it is vital to keep line of sight**

 - Prerequisites for market authorisation

 - Health Technology Assessment (HTA) for health care prioritisation

- > **Some ATMPs may treat diseases in a completely new way**

 - Healthcare systems also need to adapt

- > **HTA for ATMPs is not different than for other new technologies approaching the health care system**

 - > However, for some ATMPs the level of uncertainty is relatively high (efficacy and long-term effects) in relation to the comparator

Effects and impact. Feedback.

- The project work has led to
 - Increased knowledge and understanding
 - Collaboration and Networks
- Reduced failures in late stage in the long run

”Academy and SME rarely think of HTA but focus on research and the initial clinical trials. To early understand that there must be a prepared payer and an agreed payment model, for the patient to access the new innovative ATMPs, is becoming more and more important”

Margareth Jorvid,

Regulatory Affairs & Quality Assurance, Senior Partner of LSM group

”The report is useful as an explaining tool to be used among colleagues in-house. Very good to get insight into how it works in other countries. I am part of global corporate teams discussing exactly this”

*Joachim C Alexandersson
Market Access Manager NDD merck*

The report will widen the knowledge in all parts of our organisation. To get the full picture and understand how the different steps connect is important for all involved stake holders.

Anders Karlsson, CEO Idogen AB



-It is a great report that provides overview of reimbursement system in Sweden and other countries, practical examples, as well as challenges for utilization of current HTA models for ATMP products. Thank you!

Anna Koptina, Senior Regulatory Affairs Manager

Immunicum AB

”The examples (in the report) are relevant and illustrative. They put the spotlight on the conflict between one-year budget systems and prices aimed at covering long-term effects”

*Sofie Alverlind, Project manager,
Swedish Association of Local Authorities and Regions (SKR)*



December 2019

Thanks to a great team!

