

# FAMHP collaboration with patient organisations: STA pilots start up

FAMHP symposium on collaboration with & for the patient

BRUSSELS

25.09.2018

# Involvement of disease-specific patient organizations

## Goal: involve disease-specific patient organizations in national STA (and CTA) applications

STA = scientific technical/regulatory advice

CTA = clinical trial application

### Phase 1 (Q3-Q4 2018):

= pilot project on STA requests related to CTA's (post-hoc approach)

### Phase 2 (2019):

- STA requests related to CTA's in real-life procedures
- pilot project on CTA's



# Setup for the collaboration with disease-specific organizations

## STA Pilots: general aspects

- Q3-Q4 2018:  
5 STA procedures (related to questions for a clinical trial)/CTA assessed retrospectively
- 1 dossier per month
- Simulated assessment according to standard procedural timelines of an STA Type II/III procedure
- In collaboration with disease-specific organizations and umbrella organisations (eg. VPP, LUSS, RaDiOrg)
- Common evaluation of pilots early 2019 + next steps towards 2019 for staggered implementation in « real-life » STA procedures



# Scope of the collaboration with disease-specific organizations

**Interact with disease-specific organizations within STA procedures concerning e.g.:**

- Feasibility and design of the study proposed
- Study endpoints vs real clinical patient benefit vs survival
- Relevant patient population
- Comparator or not
- Duration of study
- Relevant patient outcomes (eg. PRO's)
- Safety concerns
- Quality of Life aspects
- Unmet Medical Needs



# List of Disease-specific patient organizations

Following criteria were set forward for the selection of the disease-specific patient organisations:

- Minimal (medical and or EU) expertise present at the level of the patient organisation (i.e. organisations for which no immediate training by the FAMHP is needed short term)
- Interest in collaboration with FAMHP in the STA pilots



# List of Disease-specific patient organizations

## Following organisations showed interest:

- HTAP voor pulmonaire hypertensie België
- BOKS: metabolic diseases
- Muco vereniging
- NET & MEN kanker
- Werkgroep hersentumoren
- 22q13 (Phelan McDermid Syndroom)
- Bindweefsel
- Crohn en colitis ulcerosa vereniging
- Hodgkin & non-Hodgkin
- LGD alliance (lymphangiomatosis & gorham's disease)
- Ligue Huntington
- Lymfklierkankervereniging Vlaanderen
- MS-liga
- RA liga (via reumanet)
- Vlaamse Parkinson Liga



# List of Disease-specific patient organizations

## Following organisations showed interest:

- Diabetes Liga
- GIRTAC: anticoagulants
- HAE: heredic angiodemia
- Association Lupus Erythématuex
- GESED: Ehlers-Danlos syndrome



# Projects selected based on the list

## 5 Pilot dossiers were selected based on:

- **Match** between interested disease-specific patient organisations and recent scientific advice requests given between 2015-2018 (post-hoc approach)
- **Topic** included: clinical trials in patients
- **Sufficient information** available in the briefing package

## General remark:

Disease area in which the interested patient organisations are active did not match well with the disease area for which the FAMHP receives STA requests and for which relevant input can be given by interested patient organisations



# Projects selected based on the list

## 5 Pilot dossiers covering:

- 4 different therapeutic areas
- 5 disease-oriented patient organisations
- 3 umbrella patient organisations (VPP, LUSS, RaDiOrg)

Pilot 1: Diabetes Type I

Patient organisation: Diabetes Liga

Pilot 2: Cystic Fibrosis

Patient organisation: Muco Vereniging

Pilot 3: Oncology

Patient organisation: Hodgkin & Non-Hodgkin; Lymfeklierkanker Vereniging Vlaanderen

Pilot 4: Ulcerative Colitis

Patient organisation: Crohn en colitis ulcerosa vereniging

Pilot 5: Diabetes Type I (paediatric population)

Patient organisation: Diabetes Liga



# Projects selected based on the list

## Pilot 1: Therapeutic Area DIABETES

**Product X: drug-device combination product**

**Medical device intended for a diffuse, extraperitoneal insulin administration (i.e. different types of commercially available insulin)**

Indication: Type 1 Diabetes Mellitus

Phase 1; First In Human study (FIH)

Study goal: to investigate safety and early performance of the Medical device in patients with brittle, prone to hypoglycemia, badly controlled Type 1 Diabetes Mellitus.

Patient population: subject of the advice request

Clinical issues:

- Target patient population – exclusion criteria
- Overall study design of the FIH study and the possibility for an extension study

Organisation of interest: Diabetes Liga



# Projects selected based on the list

## Pilot 2: Therapeutic Area CYSTIC FIBROSIS

**Product Y: a translational read-through inducing drug (TRID) for the treatment of genetic conditions caused by nonsense mutation.**

Indication: cystic fibrosis caused by nonsense mutations

Phase: 1b/2a proof-of-concept study

Study Goal: to evaluate the safety, tolerability, pharmacokinetics, pharmacodynamics, and preliminary measures of efficacy of subcutaneously administered product Y as monotherapy and in combination with product Z in nmCF patients.

Patient population: CF patients with a nonsense mutation

Clinical issues:

- CT in patient population or in healthy volunteers
- Timing of the trial in patients (in parallel with MAD study in healthy volunteers)
- Trial design
- Assessments during the trial (safety, disease marker/function, PK)

Organisation of interest: Muco Vereniging



# Practical Aspects

- Mimic real-life setting & procedural timeline
- **Timeline:** 7 weeks from receipt of the STA dossier until discussion meeting with FAMHP experts (Applicant not present in the post-hoc approach)
- **STA Dossier** = complete package as for FAMHP assessors = briefing doc (mainly non-clin/clinical part) + study protocol, Investigator Brochure (IB), list of company questions & positions  
(All documentation provided in English, no translation foreseen)
  - Unblinded, complete package for the patient organisation representatives + umbrella organisation
  - Blinded, subset of package for consulted patients
- **Intake meeting** with the involved umbrella and patient organisation to start up the procedure & provide general guidance on the FAMHP expectations (= not part of standard STA procedure)



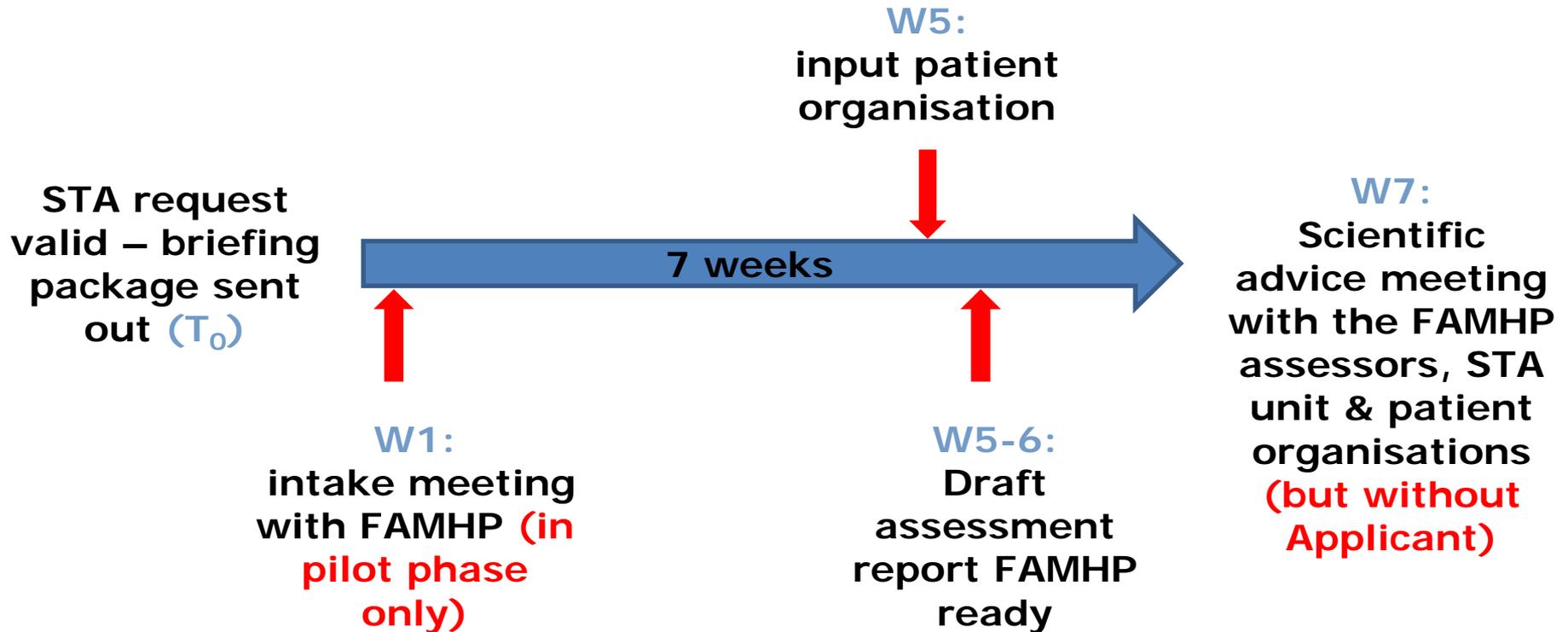
# Practical Aspects

- After 5 weeks a written advice should be provided by the patient organisations on the questions of the company, this will be given to the assessors
- Patient organisation **can formulate an advice on a selection of the list of questions of the applicant**, not all questions have to be assessed by the patient representative (FAMHP guidance provided)
- **Scope of the advice given by the patient organisation** should be limited to the topics tackled by the list of questions to respect the STA procedure (but other comments were welcomed)
- The **advice should represent the view of the patient population** and not of 1 specific patient
- The patient organisations provide their comments (in writing, in English) to the FAMHP at timepoint week 5 and receive the FAMHP assessment / advice to the Company questions at week 5-6.
- At week 7: **Discussion meeting** between the FAMHP Assesors, the STA unit and the disease-oriented + umbrella patient organisations to discuss the advices that were formulated from both sides + **feedback moment**



# Practical aspects:

Real-life setting is mimicked as much as possible:



# Practical Aspects

## GENERAL CONSIDERATIONS

- The **confidentiality** of STA dossier info should be guaranteed at all times:
  - **Representatives of patient organisations & umbrella organisations directly involved:**  
declaration of interest (DOI) & confidentiality undertaking (CU) according to current FAMHP template prior to receipt of STA dossier
  - **Consulted patient members:** internal confidentiality undertaking only
- **Written justification for the choice of representative from the disease-oriented patient organisation** was also asked
- **No specific training foreseen**, only the intake meeting to discuss the practical start-up of the STA pilot dossier and to align expectations from the FAMHP & involved patient / umbrella organisation



# First results

## GENERAL (METHODOLOGICAL) ASPECTS

- Explaining the **context & expectations** is crucial to get relevant input i.e. STA dossier, STA / CTA procedure, early-stage development aspects



Intake meeting & scientific discussion meeting = key success factors



Additional training & guidance/support may be needed (especially for less experienced patient organisations)

- Short procedural timelines = challenge
- Additional workload  creating added value
- DOI & CU sometimes considered as hurdle
- Blinding of confidential data  providing sufficient level of info



# First results (continued)

## SPECIFIC ASPECTS

- Input from patient organisations & patients is **often quite relevant and providing added value** but the **consultation process needs further improvement** on eg.
  - Timepoint(s) of consultation
  - Participation to the STA meeting with the Applicant
  - Consultation on very patient-specific questions
- **Differences in comments:**  
(semi)professional patient organisation representatives  patients  
BUT: both are important to capture !
- Comments sometimes **related to other aspects**: eg. broader development program, other indications, Informed Consent
- **Representativeness of patient's input:**  
What is the best consultation approach to insure sufficient representativeness?



# First results (continued)

## SPECIFIC ASPECTS

- Importance of **feedback moment** to the patient organisation(s)/patients  
 Training, motivation, empowerment
- Importance of **estimating / explaining the potential impact on proposed patient recommendations**:

perceived risk  innovation & patient access to new treatments

short term aspects (CTA)  long term aspects  
(MAA and reimbursement / HTA)

- **Overall outcome:**
  - promising & enriching
  - complementary to FAMHP's internal & external expert opinions
  - putting the patient at the centre



# Next steps

- Early January 2019:  
Evaluation of the 5 STA pilots + determination of the next steps to implement patient involvement in real-life STA procedures
- Call for interest:  
towards the patient organisations that were not selected for the 5 STA pilots, or other interested patient organisations to exchange information between FAMHP and the patient organisation on possible future collaboration
- FAMHP collaboration with EUPATI to facilitate training & support needs towards patient organisations



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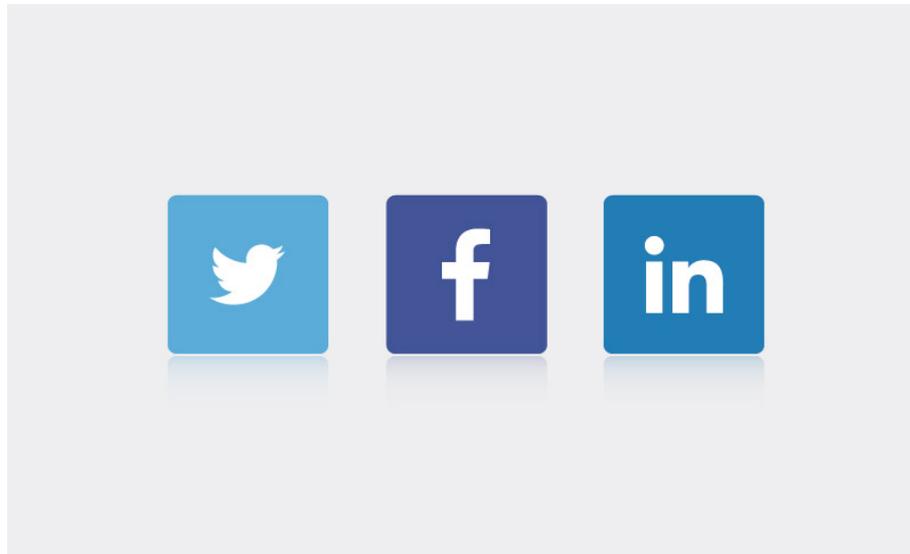
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## Acknowledgements

- Karolina Szlufcik
- Mieke Delvaye
- Greet Musch
- All participating FAMHP colleagues
- All participating & interested patient organisations
- Annemiek Van Rensen



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A large, stylized graphic of a human eye, rendered in light blue and grey tones, serves as the background for the central text. The eye is composed of a large outer arc, a smaller inner arc, and a central circular pupil area.

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