Federal agency for medicines and health products

DGPRE Supporting innovation in drug development

Greet Musch

FAMHP : SME Workshop , 2 May 2016

Current challenges in accelerating medicines development and patient access

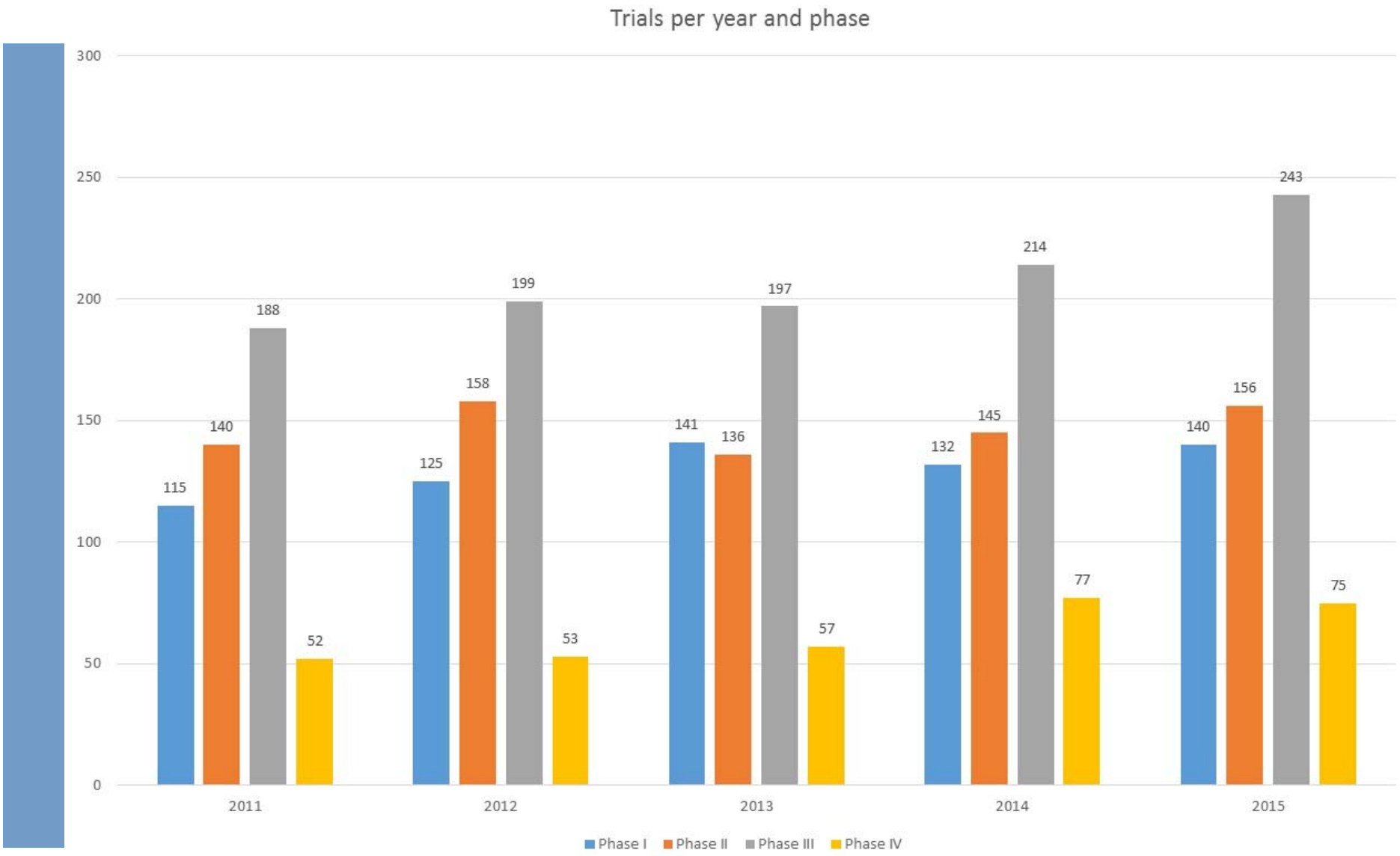
Mission of famhp as part of the EU regulatory network :

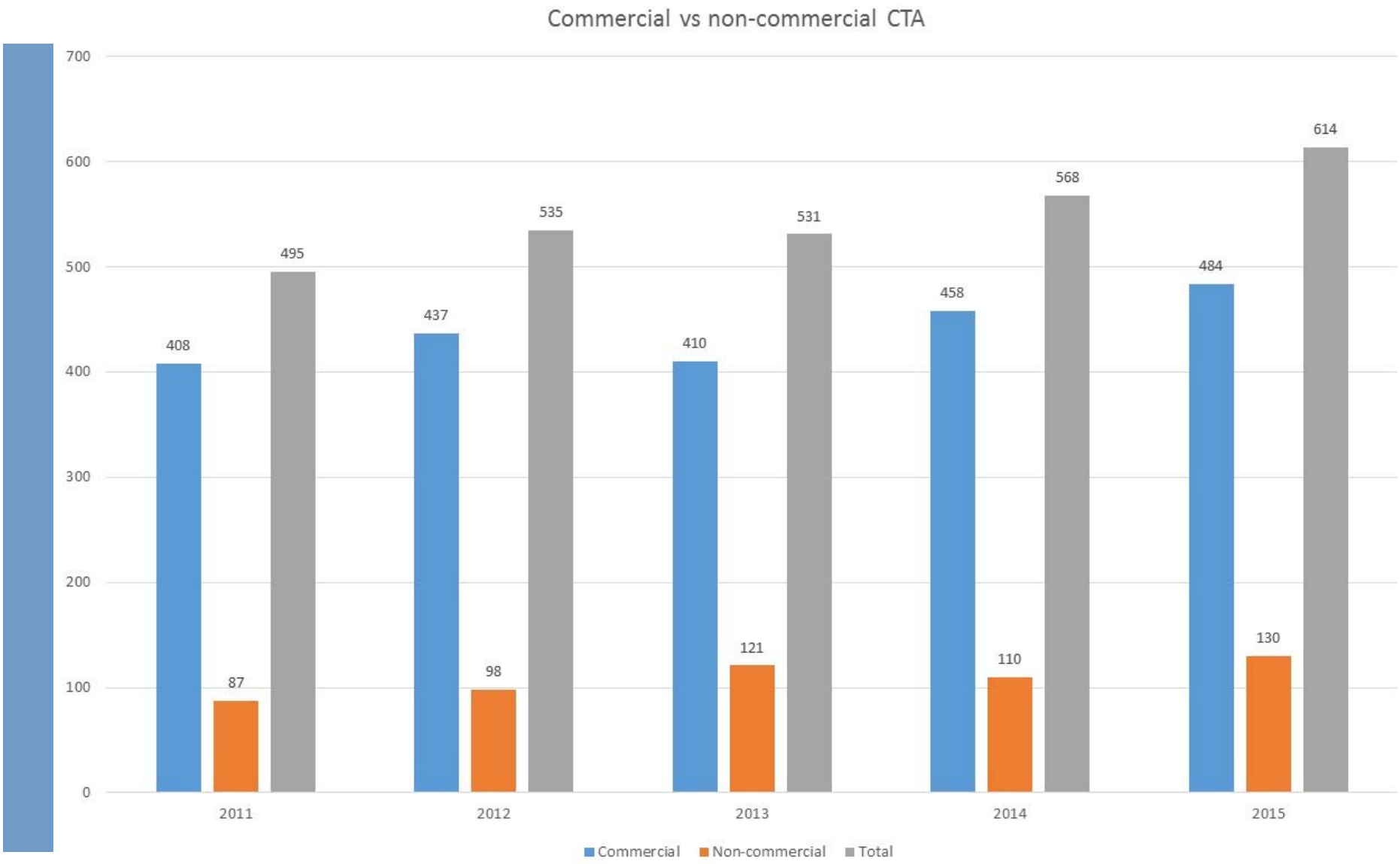
Facilitating the translation of innovative scientific advances into medicinal products meeting adequate standards and accelerate patients' access to promising therapies fulfilling unmet medical needs.

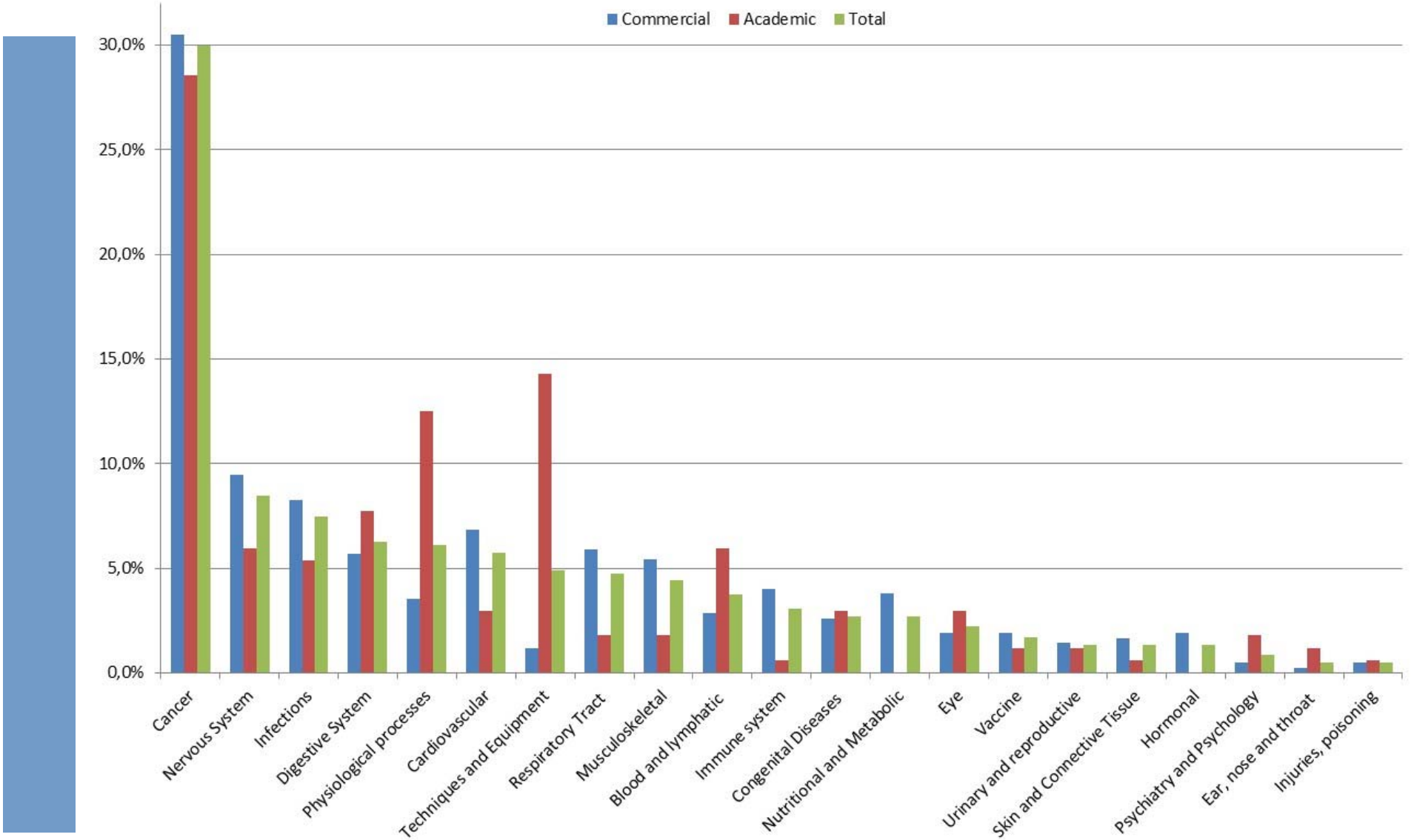
1: Current key activities : State of the art and cartography in Belgium

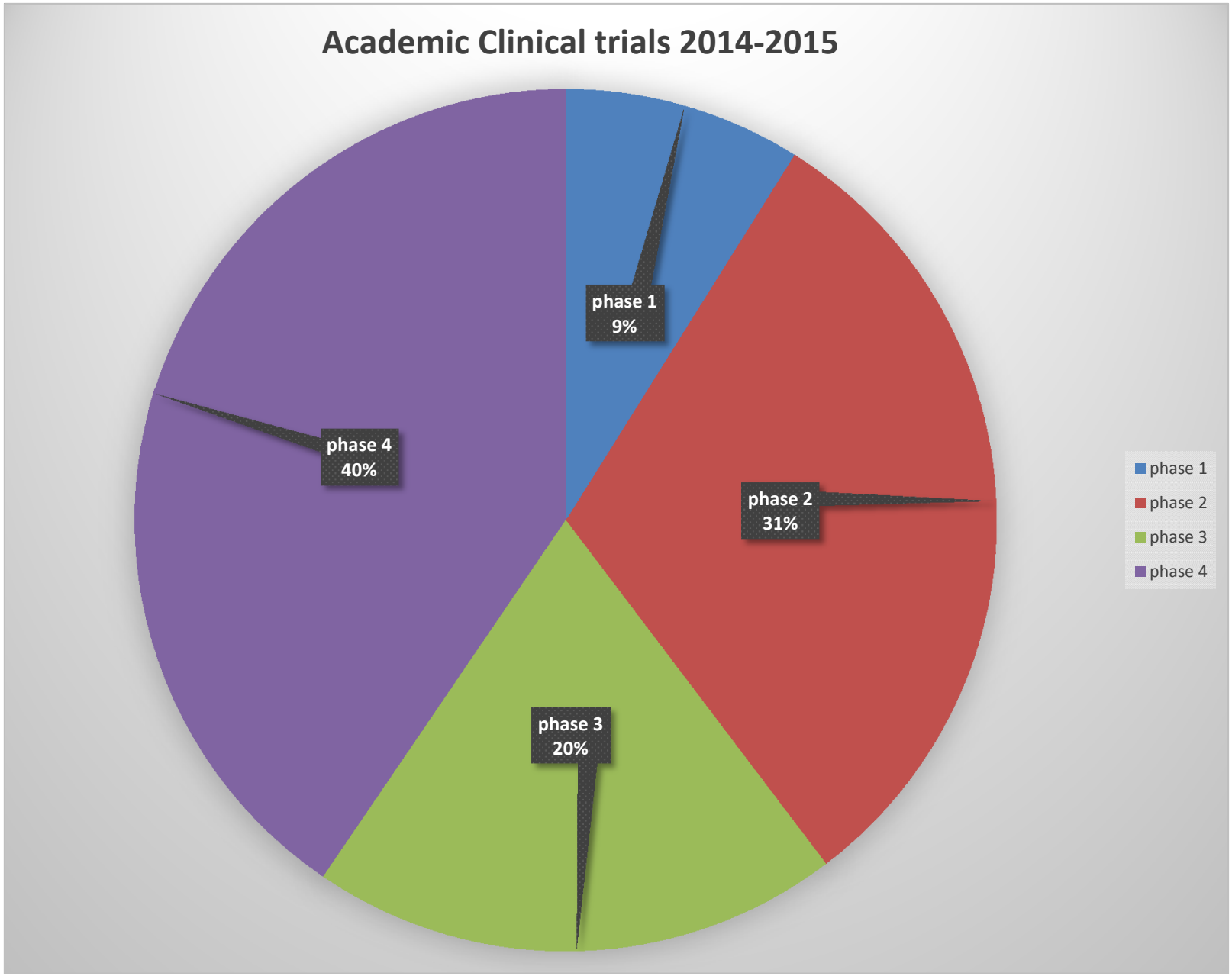
- **Clinical trials (medicinal products)**
- **Clinical investigations and evaluations (medical devices)**
- **Compassionate use and Medical Need Programs**
- **Scientific Regulatory advice at national level**
- **Scientific advice at EU level (SAWP at EMA)**
- **Marketing authorisations at EU level Rapporteurships (CHMP at EMA)**

1.1 Current key activities : State of the art and cartography in Belgium : Clinical trials

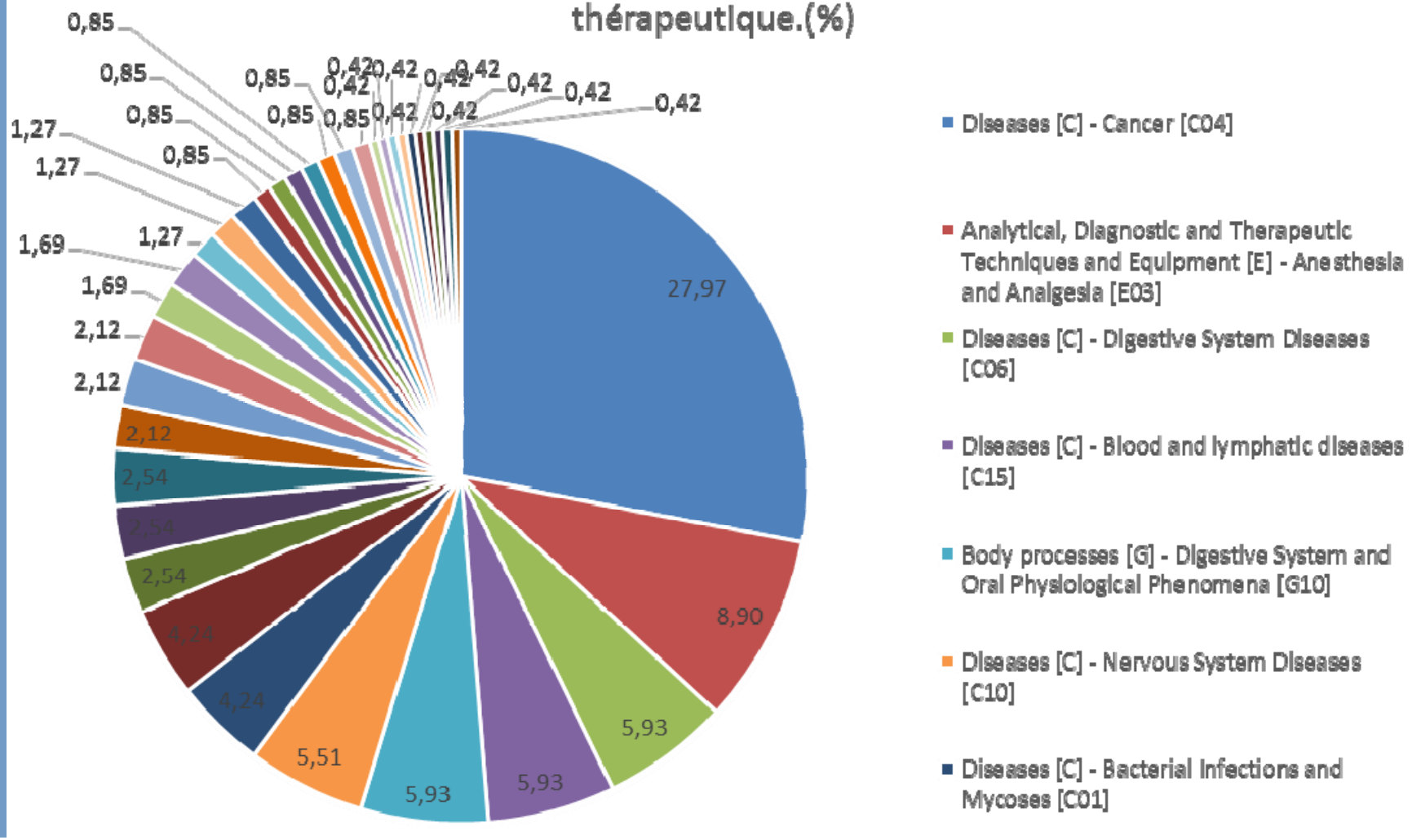

Trials per year and phase
300
250
200
150
100
50
0
115
140
188
52
125
158
199
53
141
136
197
57
132
145
214
77
140
156
243
75
2011
2012
2013
2014
2015
Phase I
Phase II
Phase III
Phase IV


Commercial vs non-commercial CTA
700
600
500
400
300
200
100
0
408
87
495
437
98
535
410
121
531
458
110
568
484
130
614
2011
2012
2013
2014
2015
Commercial
Non-commercial
Total

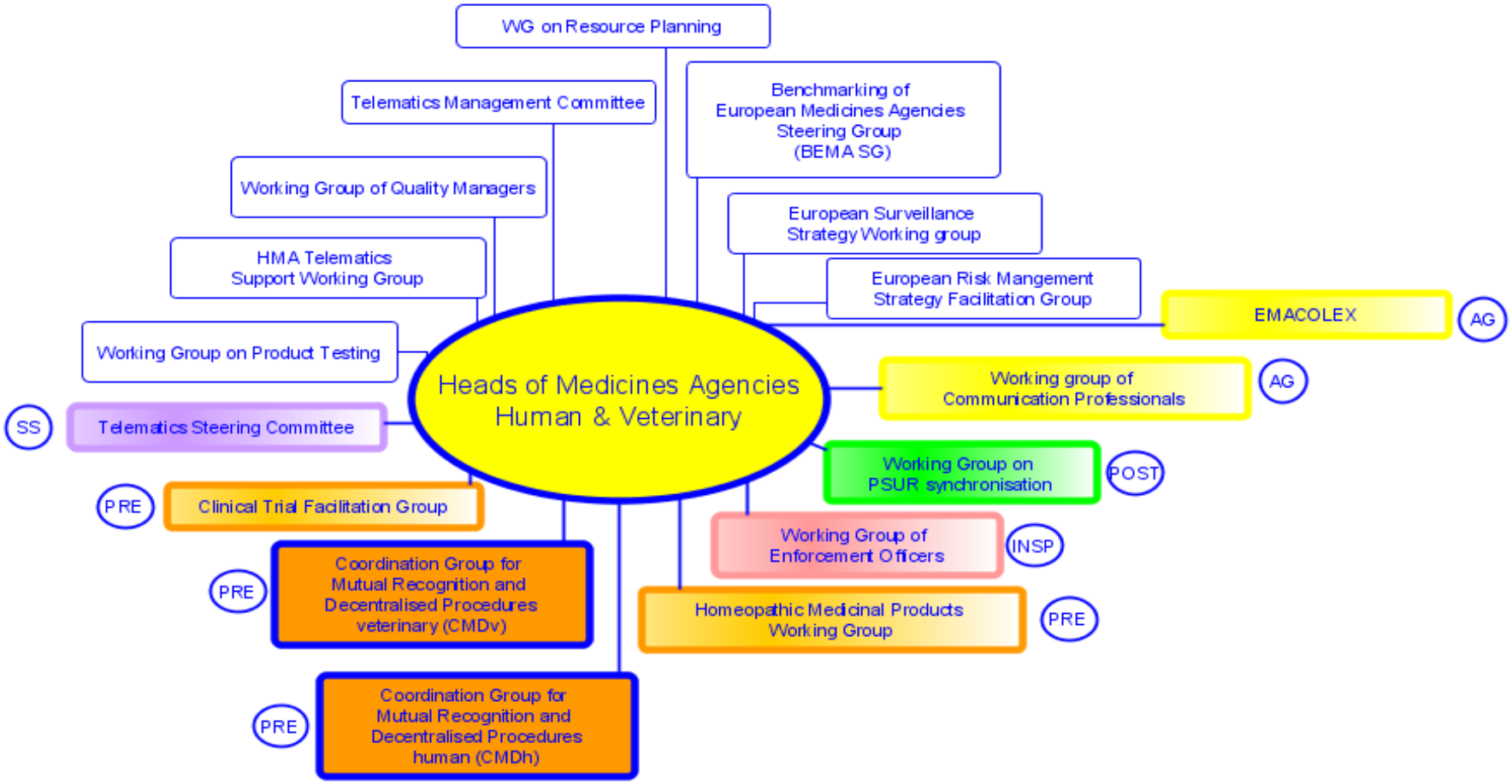

Commercial
Academic
Total
30,0%
25,0%
20,0%
15,0%
10,0%
5,0%
0,0%
Cancer
Nervous System
Infections
Digestive System
Physiological processes
Cardiovascular
Techniques and Equipment
Respiratory Tract
Musculoskeletal
Blood and lymphatic
Immune system
Congenital Diseases
Nutritional and Metabolic
Eye
Vaccine
Urinary and reproductive
Skin and Connective Tissue
Hormonal
Psychiatry and Psychology
Ear, nose and throat
Injuries, poisoning


Academic Clinical trials 2014-2015
phase 1
9%
phase 2
31%
phase 3
20%
phase 4
40%
phase 1
phase 2
phase 3
phase 4

répartition des CTA académique 2014-2015 en fonction du domaine thérapeutique.(%)

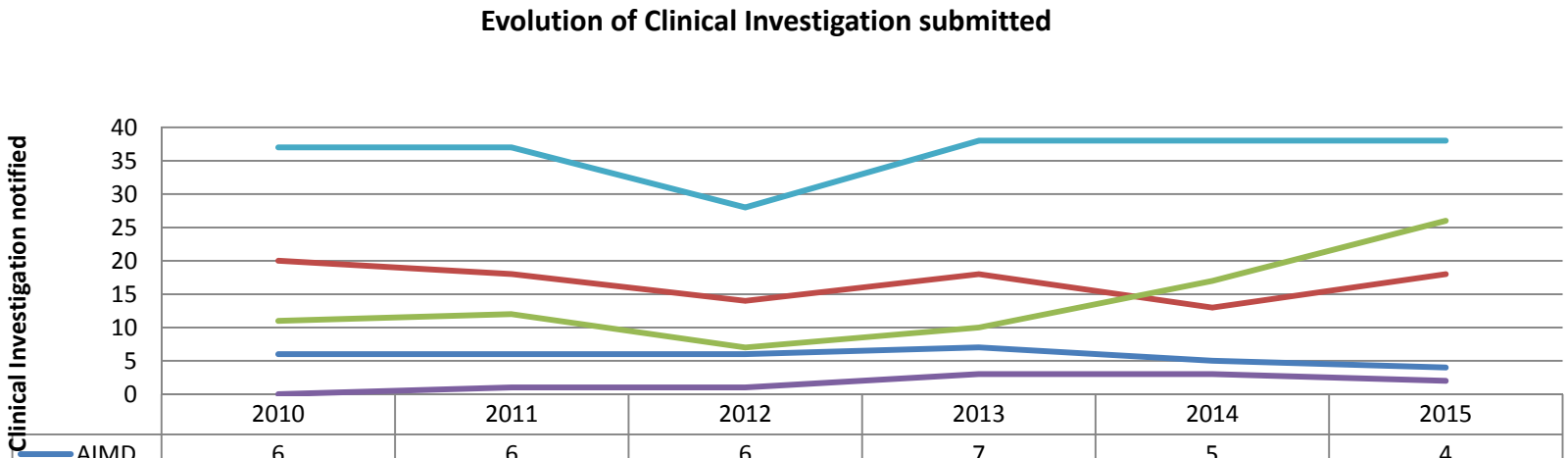


EU activities: within the HMA



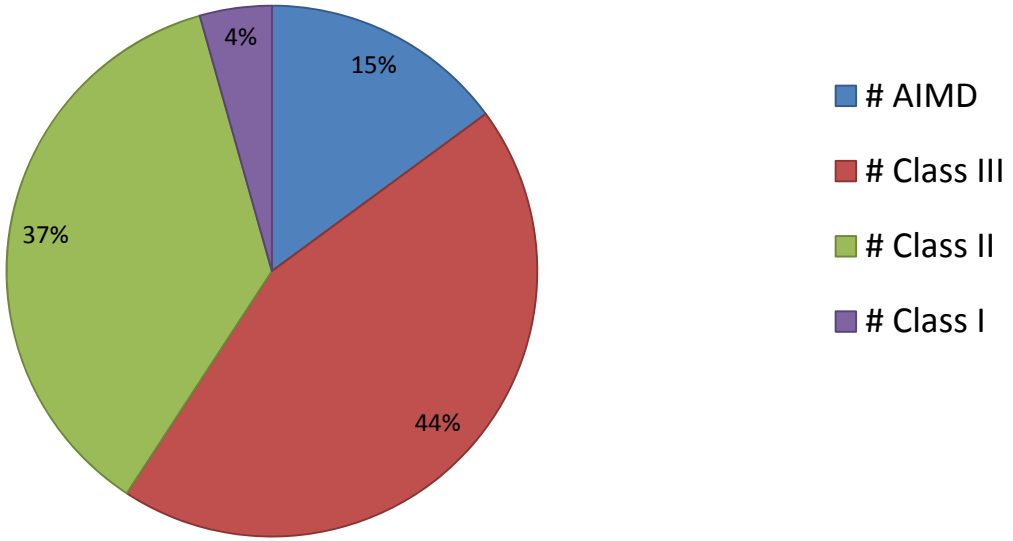
1.2 Current key activities : State of the art and cartography in Belgium :CIE

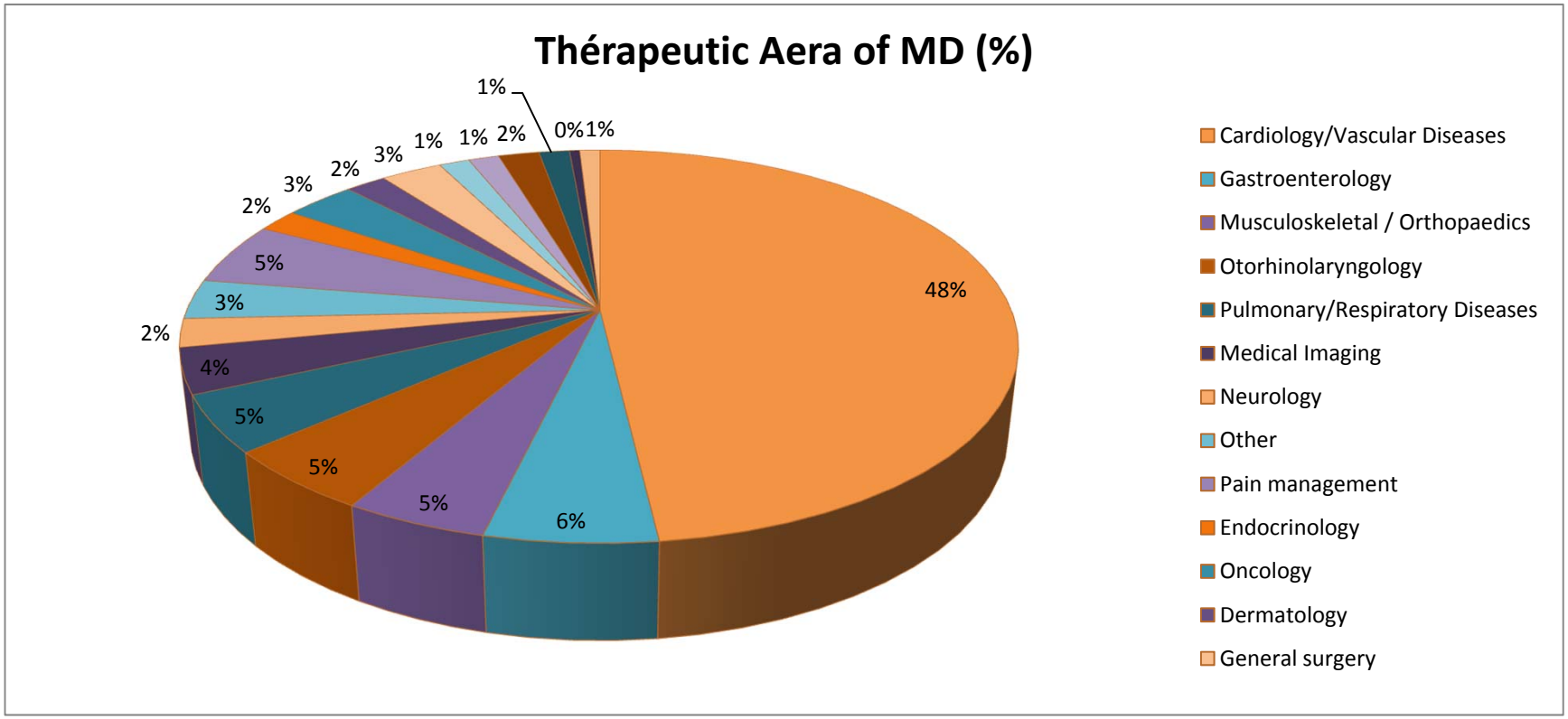
Stat meddev clinical investigation (2010-2015)



	2010	2011	2012	2013	2014	2015
AIMD	6	6	6	7	5	4
Class III	20	18	14	18	13	18
Class II	11	12	7	10	17	26
Class I	0	1	1	3	3	2
Total	37	37	28	38	38	38

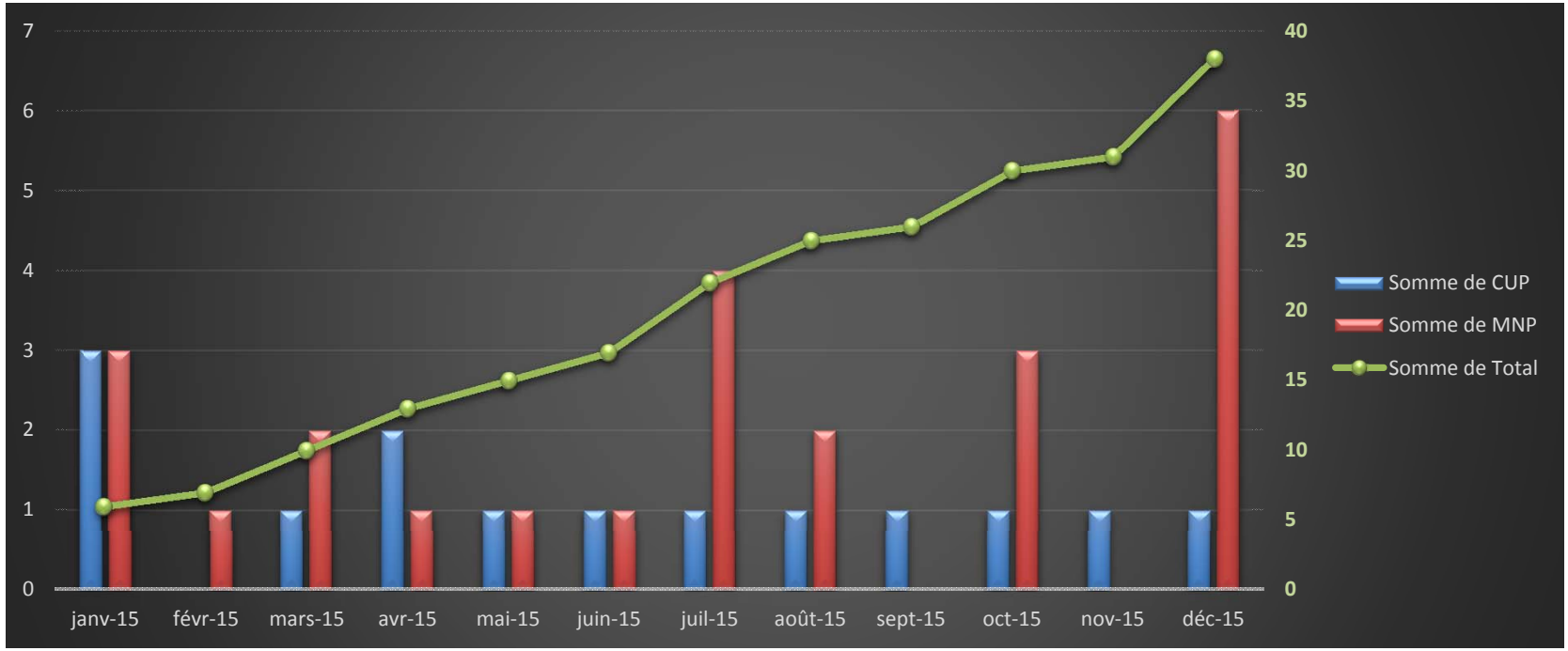
Clinical investigation distribution according to MD Class(%)



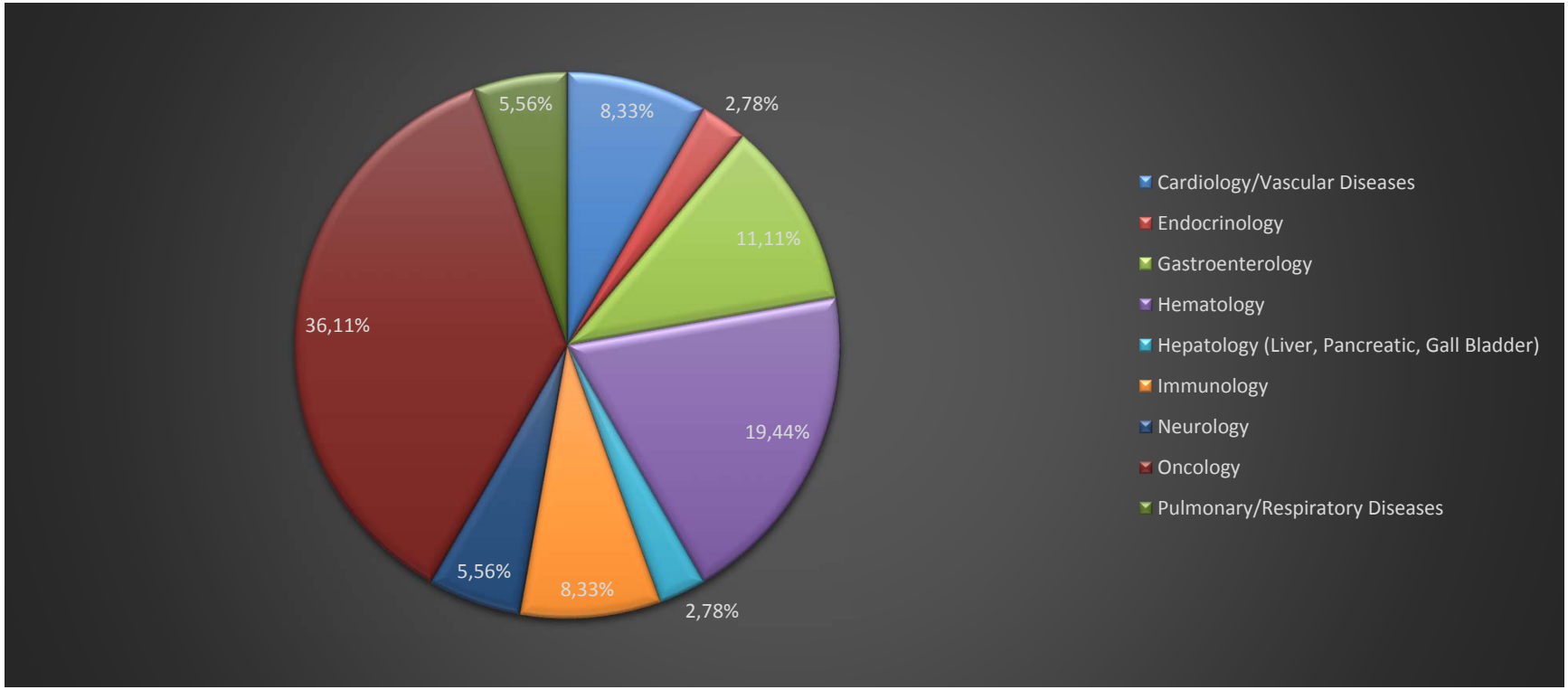

Thérapeutic Aera of MD (%)
48%
6%
5%
5%
5%
4%
2%
3%
5%
2%
3%
2%
3%
1%
1%
2%
0%
1%
Cardiology/Vascular Diseases
Gastroenterology
Musculoskeletal / Orthopaedics
Otorhinolaryngology
Pulmonary/Respiratory Diseases
Medical Imaging
Neurology
Other
Pain management
Endocrinology
Oncology
Dermatology
General surgery

1.3 Current key activities : State of the art and cartography in Belgium : CU and MNP

UMN


7
6
5
4
3
2
1
0
40
35
30
25
20
15
10
5
0
janv-15
févr-15
mars-15
avr-15
mai-15
juin-15
juil-15
août-15
sept-15
oct-15
nov-15
déc-15
Somme de CUP
Somme de MNP
Somme de Total

Therapeutic Area (CUP and MNP 2015)



1.4 Current key activities : State of the art and cartography in Belgium : Scientific advice

Figures for the Scientific-Technical Advice & Knowledge Management unit

Number of National Scientific-Technical Advices

		2014 IN	2014 OUT	2015 IN	2015 OUT
Totaal aantal nationale WTA-aanvragen		**33**	**35**	**41**	**45**
According procedure	Type III WTA	20	20	26	26
	Type II WTA	11	12	6	9
	Type I WTA	2	3	9	10
According (spearhead) domain	VACCINS	9	10	11	13
	EARLY PHASE DEVELOPMENT	14	13	21	20
	ONCOLOGIE	7	8	4	5
According use	Menselijk	33	35	41	45
	Diergeneeskundig	0	0	0	0

Number of European Scientific-Technical Advices

		2014 IN	2014 OUT	2015 IN	2015 OUT
Totaal aantal Europese SAWP adviesaanvragen		**86**	**86**	**93**	**91**
According procedure	Scientific Advice	81	81	92	90
	Qualification procedures en HTA	4	4	1	1
According (spearhead) domain	VACCINS	4	6	9	9
	EARLY PHASE DEVELOPMENT	1	2	8	8
	ONCOLOGIE	24	25	13	13
	BIOSIMILARS	N/A	N/A	8	8

1.5 Current key activities : State of the art related to SAWP

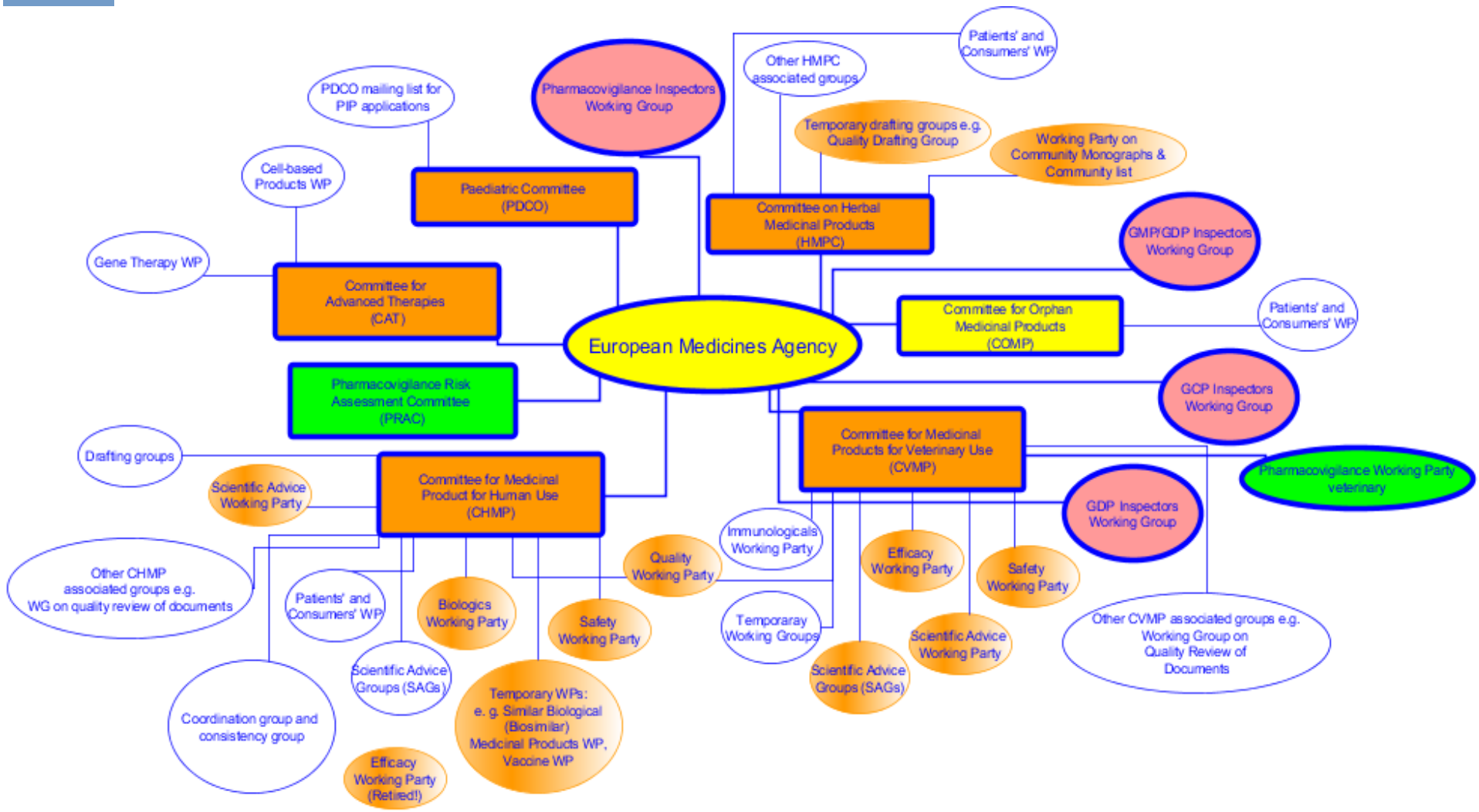
- **EPD: keeping short timelines, started training with stakeholders, internal endorsement and harmonisation of grounds for non-acceptance, start of communication plan (internal and external), preparation of accreditation of fase I units conducting first in human trials.**

- **SAWP: coordinator for 92 advices on a total of 472 (not including //HTA advices), of which 8 early phase projects, 9 vaccines, 13 in oncology. One of the early phase advices was also in oncology.**

- **PRIME and adaptive pathway projects: active involvement in eligibility. Coordinator for 1 //HTA advice.**

1.6 Current key activities : State of the art related to Rapporteurships at CHMP

EU activities: within the EMA



1.6 Current key activities : State of the art related to Rapporteurships at CHMP

Appointed in 2015

7 Rapporteurships + 1 Peer-reviewer

Medicines used in diabetes

Glucagon (Rap)

Empagliflozin/Linagliptin (CoRap)

Lixisenatide/Insulin glargine (CoRap)

Insuline glargine (Peer Reviewer)

Antineoplastic medicines

Paclitaxel (Rap) – Hybrid application (new indication)

Lenvatinib (mesilate) (Rap)

Vaccines

Dengue vaccine (Rap)

Immunosuppressants (Antirheumatic)

Baricitinib (CoRap)

The FAMHP "spearhead domains"

The FAMHP wants to <u>invest</u> and <u>excell</u> in the following « spearhead domains »

- Vaccines
- Oncology
- Early phase development (W. Janssens)

1.7 Current key activities : State of the art related to Vaccines

- **Coördinator appointed from 18 th April 2016**

- **Priorities focused on :**
  - **Cartography of activities / initiatives at (inter)national and regional level**
  - **Gap analysis expertise**
  - **Optimisation of activities at famhp (R&D-MA-Vaccinovigilance – GXP Inspections- Proper use etc)**
  - **Facilitation of recruitment of healthy volunteers in clinical trials and and follow – up .**
  - **Communication plan : Seminar Q3 2017**
  - **Financing (cfr pilot EPD)**

2 : New evolutions and initiatives (2/1)

- Implementation of the New Clinical trial regulation

National legislation impacted by CTR 536/2014

- Ethics committee (EC) organisation/interaction with FAMHP
- Single decision
- Exceptions from Manufacturing/Import Authorisation (MIA)
- Single fee
- Language

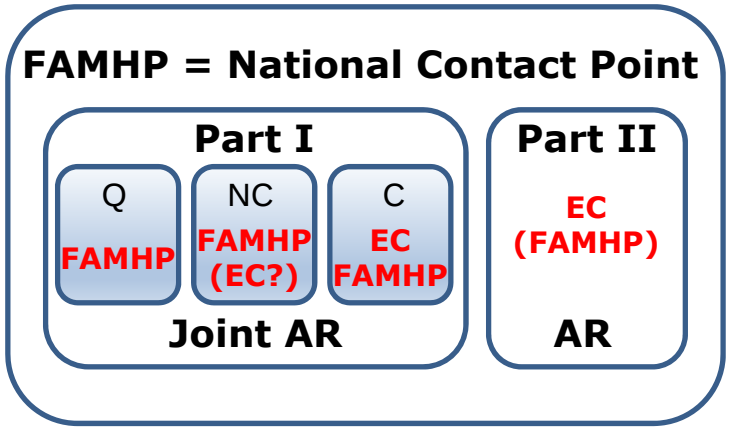
FAMHP & EC Organisation

- **Principle of "joint assessment" : both instances can take a position on all points described in Part I/II**
- **Definition of « primary » responsibility that needs to assess, document and discuss these aspects on the EU level**
- **Some points remain the responsibility of the 2 instances, with different philosophies**

Alignment at national level & next steps (1)

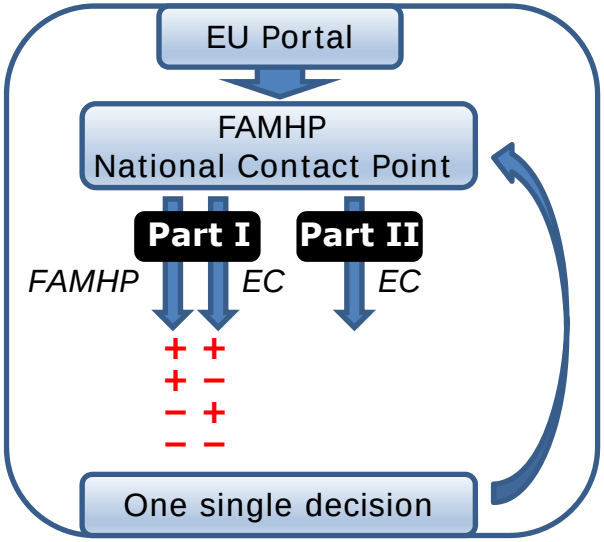
1. FAMHP = National Contact Point

AR Part I:	**Quality:**	**FAMHP**
	Non-Clinical:	**FAMHP (EC?)**
	Clinical:	**EC and FAMHP**
AR Part II:		**EC (FAMHP)**



Alignment at national level & next steps (2)

2. **Evolution towards national Ethical Review Board ("Umbrella" / "College") in a one-to-one relationship with FAMHP**



3. **Independent ethical evaluation**
4. **Representation of lay-men / patients**
5. **Added value of co-assessment (touch with medical reality)**
6. **Short timelines for phase 1 trials will be maintained**
7. **Pilot joint assessment FAMHP-EC foreseen early 2017**

2 : New evolutions and initiatives (2/2)

- **Revisiting the current procedures for marketing authorisations at CHMP :**
  - **Conditional marketing authorisation**
  - **Accelerated assessment**
  - **Prime**
  - **Adaptive pathways**
- **EU network of national innovation offices : bridging the gap between research at national level and promising new innovative medicinal product fulfilling an unmet medical need**

2 : New evolutions and initiatives (2/3)

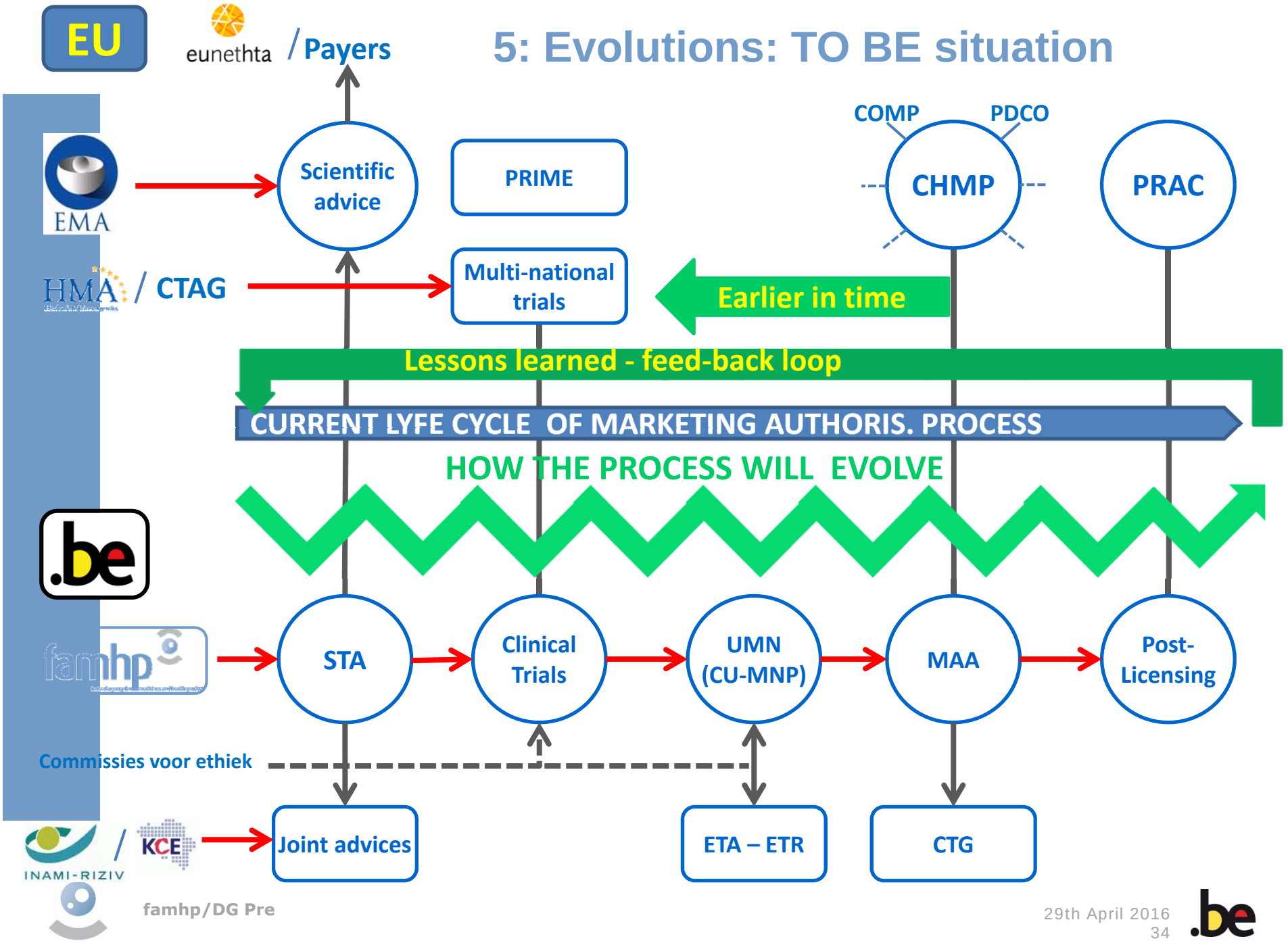
- **Others :**

  - **Repurposing established medicines/active substances**
  - **Real World Evidence**
  - **Personalised medicines**
  - **Efforts harmonising HTA at EU level , involving payers at an early stage , in collaboration with Licensing Agencies**
  - **…**

2: New orientation in view of evolutions EU (2/4)

- **Focused activities in Safe and Timely Access to Medicinal products**
- **Optimised EMA processes on conditional MA and accelerated assessment**
- **Active engagement of famhp in PRIME (target 2 Rapporteurships in 2016)**
- **Repurposing active substances/ medicinal products**
- **Enhancing joint advices with HTA bodies**
- **Installing the national innovation office as part of the EU network (bridging the gap preclinical-elegibility for PRIME**

5: Evolutions: TO BE situation



Thank you for your attention

Your medicines and health products, our concern

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.be