Belgian Symposium on the
Integration of Molecular Biology Advances
into Oncology Clinical Practice

Access of Belgian cancer patients to therapeutic innovations

P. Neven ; K. Punie

MBC, UZ Leuven

December 2-3 2022

Access of Belgian cancer patients to therapeutic innovations

“No cost to patient”

Focus on breast cancer

COI december 2022

- 100% employed UZ en KU-Leuven
- Werkgroep/ Groupe de travail ‘oncologie’ CTG
- No personal financial interests
- Institutional financial interests for
  - Steering Committee, Consultancy,
  - Speakers fee, Ad Board, Travel Grant
  - This presentation

Why me?

Previously involved in reimbursement innovative cancer therapies

Non-medication

- SLN (took 3 years)
- Tumor localisation (quick)
- Reconstr. breast surgery (convention)
- Multigene assays MP/Odx
- (convention)
- High risk/ breast imaging
- ….

Medication prior to authorization (2014)

- Adj Trastuzumab 2005
  - EMA ABC: Aug 2000
  - Oct 25, 2005 (Art 56* conventie)
  - EMA EBC: May 2006
  - RIZIV/INAM: Jul 2006
    - Breast Clinics→All centers

KB 25-3-1964 →Article 106-109 KB 14-12-2006→ 25-04-2014

→2022: large increase in requests from physicians→ pharma for oncology indications

Medications : very long way from lab → patient prescription

577d + extra days EMA-CTG … if reimbursed…

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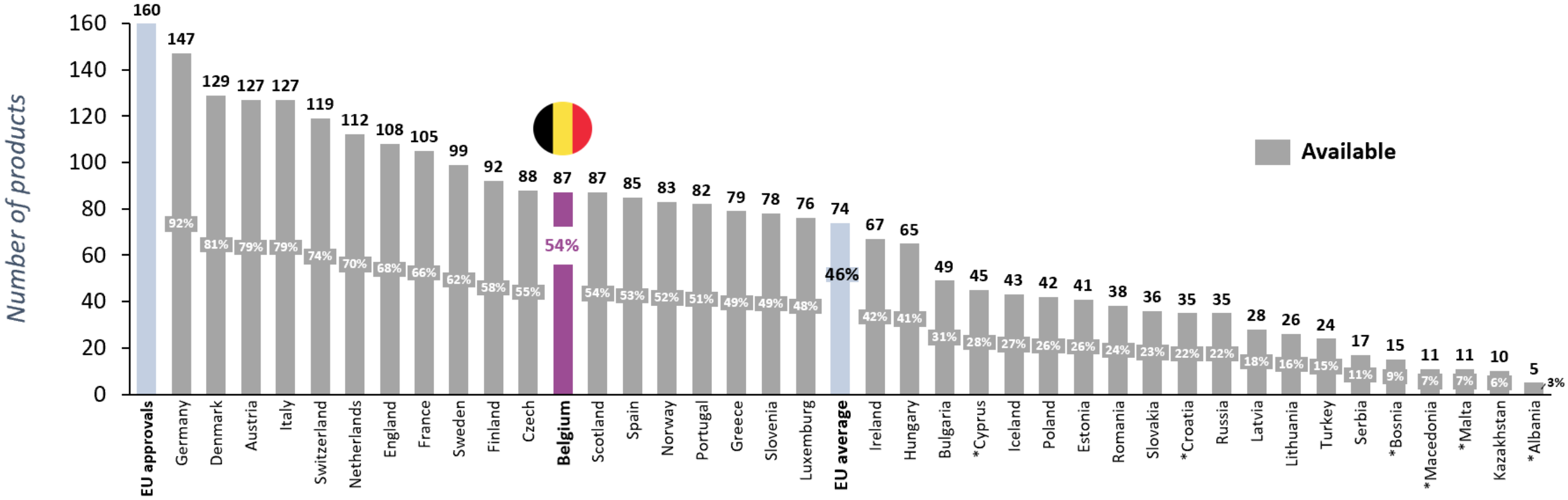
Only 54% of authorized drug is reimbursed in Belgium

pharma.be

EFPIA PATIENTS W.A.I.T. INDICATOR 2021 SURVEY

How many of the 160 EMA registered drugs (2017 – 2020), available 1 jan 2022?



Source: https://www.efpia.eu/media/636821/efpia-patients-wait-indicator-final.pdf

Belgische patiënten wachten te lang op nieuwe geneesmiddelen

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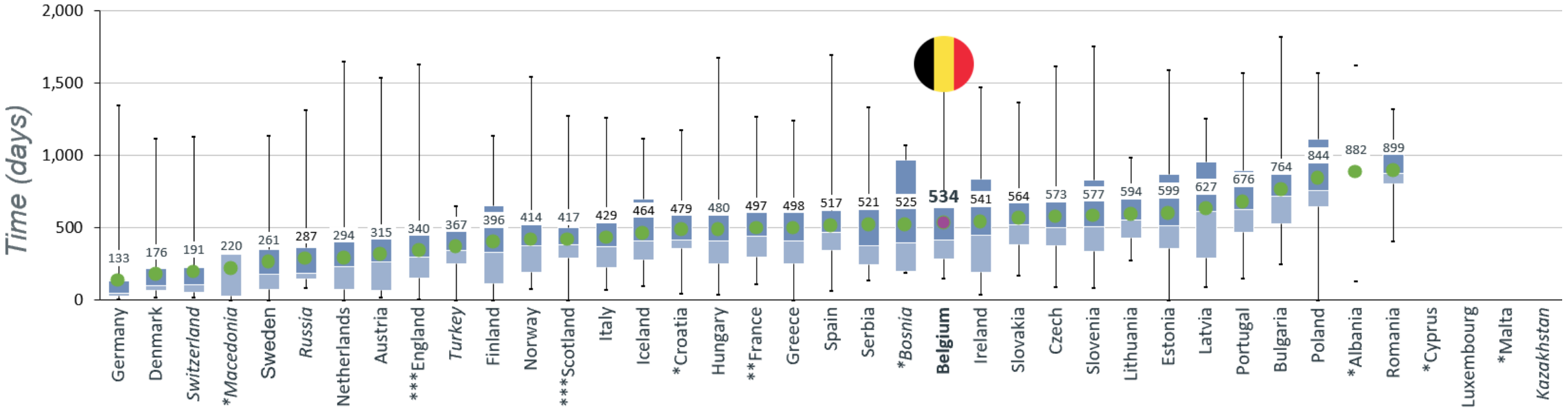
EFPIA PATIENTS W.A.I.T. INDICATOR 2021 SURVEY

How many days between EMA market authorization (2017-2020) and availability?



Source: https://www.efpia.eu/media/636821/efpia-patients-wait-indicator-final.pdf

Some “recent” examples of delays in metastatic breast cancer with a high need

DRUGS FOR ADVANCED BREAST CANCER	FDA	EMA	REIMBURSED IN BELGIUM
Atezoliz + nab-paclitaxel	Mar 2019	Aug 2019	1-4-2021
Olaparib	Jan 2018	Apr 2019	1-4-2022
Talazoparib	Oct 2018	Jun 2019	1-7-2021

Current problems to early access anti-cancer therapies

Reimbursement Commission

- Long waiting time until reimbursement for tested indication
  - Waiting EMA approval → scientific evaluation …repeated by INAMI/RIZIV/CTG/CRM …
  - European administration is inefficient, cumbersome process
- Lack of transparency and methodological validity in reimbursement procedure
  - No clear framework for the scientific evaluation by CTG/CRM ⇔ Dutch BOM PASKWIL criteria
  - Scientific interpretation by internal experts is often divergent from guidelines and clinican's preference
  - Scientific and financial arguments are often confused and/or intertwined
  - Errors in scientific evaluation reports and sometimes in indication proposals

- Industry-related issues
  - Financial toxicity of anti-cancer R/
  - Not all companies ask for reimbursement in Belgium (small country, long costly procedures)
  - Legal framework hampers reimbursement procedures for combined R/, especially if more companies involved
  - Ability to start early access programs strongly directed by global pharma, not by request of physicians
  - Differences in willingness to approve individual medical need requests
- Only EMA-approved indications considered for reimbursement ⇔ T. Agnostic FDA approvals (e.g. TMB, MSI-H)
- Diagnostic issues
  - Required diagnostic test not reimbursed

...Other Problems...

...Reimbursement more restrictive than label (Chapter 4)

EMA registratie voor:

- ✓ **ernstige** graad van tumor **X**
- ✓ ~~minder ernstige~~ graad van tumor **X**
- ✓ **volwassen**
- ✓ **tabletten**
- ✓ **1x per dag 100 mg**

Not reimbursed

Waarop kunnen deze voorwaarden betrekking hebben?

Op de patiënt

- ✓ Niet-gezondheid gerelateerde kenmerken: geslacht *(man, vrouw)*, leeftijd *(kinderen, jonger dan 18 jaar, volwassenen, ouderen)*
- ✓ Gezondheid gerelateerde kenmerken *(menopauze, nierinsufficiëntie, (over) gewicht (BMI), ...)*

Op de voorschrijver

- ✓ Arts-specialist in een bepaalde discipline of met een bijzondere bekwaamheid
- ✓ Arts verbonden aan een referentie- of expertise centrum
- ✓ Arts verbonden aan een universitair ziekenhuis

Op de ziekte of pathologie

- ✓ Precieze omschrijving van de ziekte *(die beperkter kan zijn dan de ziekte vermeld in de bijsluiter van het betrokken geneesmiddel)*
- ✓ Ernst van ziekte (*matig, ernstig, vergevorderd, gemetastaseerd, ernst bepaald in functie van bepaalde erkende scores)*
- ✓ Testresultaten of rapporten *(of multidisciplinair Oncologisch Consult)* die moeten voorgelegd worden die de aanwezigheid of de ernst van de ziekte moeten bevestigen

Op de behandeling

- ✓ In overeenstemming met *(internationaal)* erkende richtlijnen
- ✓ In eerste, tweede of derde lijn wanneer eerdere behandelingen niet of niet meer werken of wanneer andere behandelingen tegen-geïndiceerd zijn.
- ✓ In combinatie met andere geneesmiddelen of behandelingen
- ✓ Onverenigbaarheden met andere geneesmiddelen of behandelingen
- ✓ Maximaal te gebruiken doseringen
- ✓ Verplichting om de behandeling te stoppen wanneer ondanks de behandeling de ziekte verder evolueert *(stopping rules)*

À quoi ces conditions peuvent-elles s'appliquer ?

Au patient

- ✓ Caractéristiques non liées à la santé : *sexe (homme, femme), âge (enfants, jeunes de moins de 18 ans, adultes, personnes âgées)*
- ✓ Caractéristiques liées à la santé : *(ménopause, insuffisance rénale, (sur)poids (IMC), etc.)*

Au prescripteur

- ✓ Médecin spécialiste d'une discipline spécifique ou ayant une formation particulière
- ✓ Médecin lié à un centre de référence ou d'expertise
- ✓ Médecin lié à un hôpital universitaire

À la maladie ou pathologie

- ✓ Description précise de la maladie (*qui peut être plus limitée que la maladie indiquée dans la notice du médicament en question)*
- ✓ Gravité de la maladie *(modérée, grave, très avancée, métastatique, gravité déterminée en fonction de certains scores reconnus)*
- ✓ Résultats de tests ou rapports *(ou consultation oncologique multidisciplinaire)* qui doivent être présentés et confirmer la présence ou la gravité de la maladie

Au traitement

- ✓ Traitement conforme à des lignes directrices *(internationales)* reconnues
- ✓ Traitement de première, deuxième ou troisième ligne à dispenser quand les traitements précédents ne fonctionnent pas ou plus ou quand d'autres traitements sont contre-indiqués.
- ✓ En combinaison avec d'autres médicaments ou traitements
- ✓ Incompatibilités avec d'autres médicaments ou traitements
- ✓ Dosages maximaux à appliquer
- ✓ Obligation d'arrêter le traitement s'il ne permet pas d'enrayer la progression de la maladie *(« stopping rules »)*

Olaparib metastatic	Jan 2018	Apr 2019	1-4-2022

But reimbursement restricted to TNBC ⇔ drug label

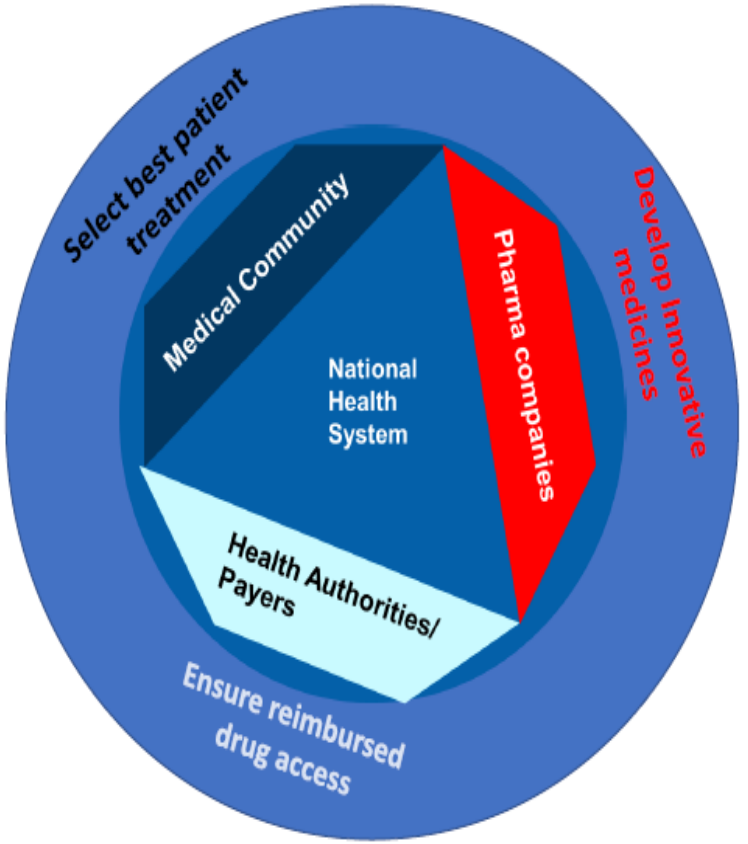
Today's Topic: Early access to therapeutic innovations "without cost"

- Drugs in **Reimbursement Chapter I**
  - Off label = OK (no restriction/ no ctrls)
- Clinical **Trials** (commercial; IIT; basket)

No label

- **Prior to** Registration
  - Compassionate Use Program (CUP*).

- **After** Registration (prior to reimbursement)
  - Medical Need Program (MNP*)
  - Off label beyond CUP/MNP **(with cost)**
  - Special Solidarity Fund
  - Free drug samples (8 per physician per year)
  - Individual Urgent Medical Need*



*Cost for company; contract negotiations to avoid patient costs

Different ways to access innovative anti-cancer drugs: Clinical Trial

pharma.be

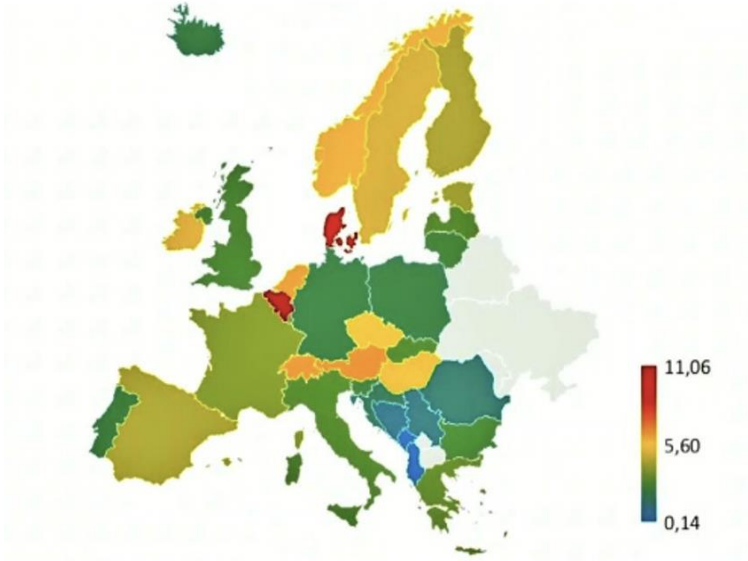
Access prior to European marketing authorization ('early access')

- clinical trials
- Compassionate Use & Medical Need Program

→Risk/Benfit by FAGG/AFMPS 'efficacy; quality; safety;

Access after European marketing authorisation

- via terugbetaling
- andere wegen



Carneiro A., et al. Annals of Oncology 2020 (31s4)

n of oncology clinical trials / 100k inhabitants (2009-2019)

We are a hub of clinical trials

Outside a clinical trial: CUP/MNP

Belgian law* describes conditions for access to medications, not authorized or different indications
(unmet medical need or a major therapeutic advantage)

Non- authorized drugs: **compassionate use** for pts with a chronically, seriously debilitating or life- threatening disease (*) ; no authorized medicinal product available. The medicinal product concerned must either be subject of application marketing authorization by the centralized procedure or must be undergoing clinical trials for related indication.

Authorized in Belgium (other indication): pts with (*) that cannot be treated satisfactory by a product that is authorized for this indication (and commercially available) in Belgium ("**medical need programs**").

UNFORTUNATELY under 3 conditions :
o a demand to obtain authorization for this indication in question is in process
o or **indication authorized but product not commercially available**
o or clinical trials ongoing in this indication

dossier to be submitted to afmps/fagg
→max duration …60d to obtain drug

*The law on unauthorized medicinal products (25-3-1964); 2004/2006/2016

How to obtain – without cost- innovative therapies? Belgian Law: milestones 2004/2006/2016? CUP/MNP/ETA

- **2004**: EU regulation allowing pharma companies to provide drugs with no marketing authorization for patients with life threatening diseases through ***CU(P)***

(initiated by company months prior to market access)

- **2006**: Belgian HA* extends EU regulation to include ***MNP***, allowing pharma companies to provide drugs already on the market free of charge, usually for off-label indications

All CUP/MNP programs (submitted after 01/07/2014) on web site: 'authorized programs', 'closed programs' or 'on hold programs'. For each of these programs: 'Summarized information for publication' + approved 'ICF'

How can we, physicians motivate companies to start more such programs?

Some “recent” examples of delays in metastatic breast cancer with a high need

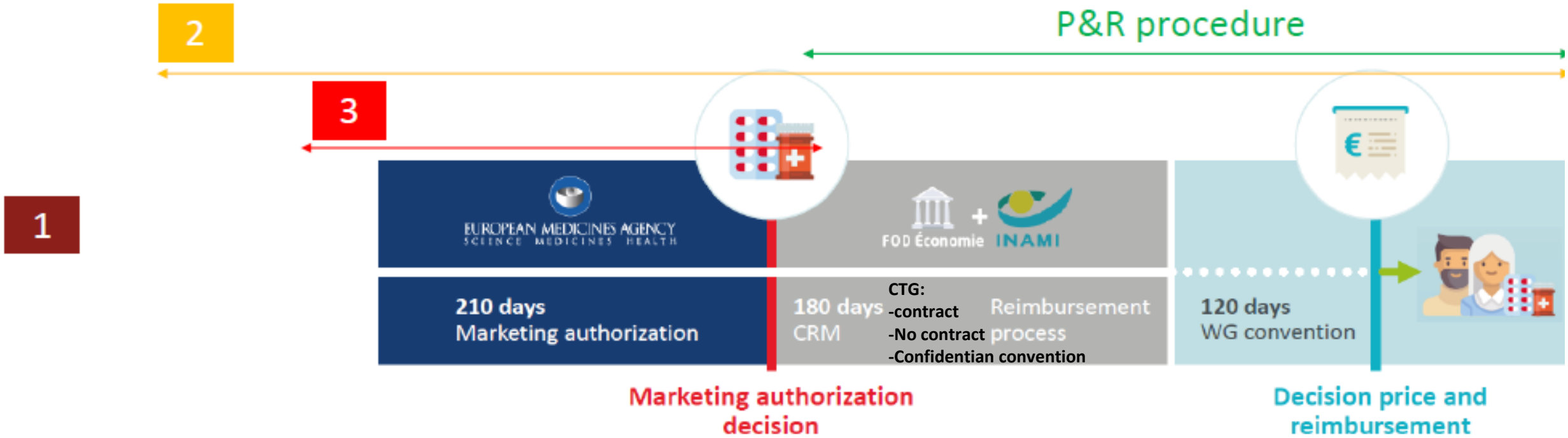
DRUGS FOR ADVANCED BREAST CANCER	FDA	EMA	FAGG MNP/CU/ETA/ samples	REIMBURSED IN BELGIUM
Atezolizumab + nab-paclitaxel	Mar 2019*	Aug 2019	v	1-4-2021
Olaparib	Jan 2018	Apr 2019	v**	1-4-2022
Talazoparib	Oct 2018	Jun 2019	v	1-7-2021

* Atezolizumab is no more FDA approved in this indication
** MNP discontinued before reimbursement was granted

Adjuvant Olaparib

Phase III OlympiA (n=1836) *gBRCAm* HER2-neg : 1 jr Olaparib (300mg BID) vs plac
Very High Risk pts → ADJ LYNPARZA 1jr →4-jr
+3.4% OS; HR=0.68; [98.5% CI, 0.47-0.97]; p=0.009. Ann Oncol Oct 2022
EMA Authorization July 2022

Procedures: general overview



Unmet Medical Need process consist of 3 steps:

1. Listing the indication on the **Unmet Medical Need list (UMN)** – at any time (> 1y before ETA request, approved only once a year)
2. **Early Temporary Authorization (ETA) - CUP and MNP framework**: at any time after UMN (60 days procedure)
3. **Early Temporary Reimbursement (ETR)**: earliest 6m before EMA submission AND mandatory reimbursement request 6m after EMA approval (90 days procedure)

Belgian Law 2016: Once the CUP or MNP is approved → ETR can be done at the RIZIV/INAMI: GDPR unprove…

‘off label’ use… (as defined by package insert).

No registration/ KCE report 2015; 252A/

Another indication, another pt group, another dose, dose interval,another route of admin.

Off –label use is possible, allowed (Belgian law), *needed: if no Clin Trial/MNP/CUP inform patient (uncertainty regarding efficacy and risk). A prescription inconsistent with the conduct of a reasonable and prudent professional practitioner would be a fault under Belgian law which could trigger medical practitioner’s liability. Cost!

***Evidence based; guideline + Patient Informed**

No budget in Belgium for off-label treatment indications / early access

EMA registratie voor:

- ☑ **ernstige** graad van tumor **X**
- ☑ **minder ernstige** graad van tumor **X**
- ☑ **volwassen**
- ☑ **tabletten**
- ☑ **1x per dag 100 mg**

EMA keurde geneesmiddel *niet* goed voor:

- ☒ **zeer ernstige** graad van tumor **X**
- ☒ ernstige graad van tumor **Y**
- ☒ **kinderen** (<18 jaar)
- ☒ **siroop**
- ☒ **2x per dag 50 mg**

off-label use could be unlawful if this is not done with the usual care

. Don’t abuse therapeutic freedom (Orde Der Artsen)

IV. Special Solidarity Fund
Financial Intervention for 1 pt for expensive drug

→Individual off-label requests: could the SSF intervene?

No Way!

- SSF recognizes that individual off-label requests is a gap in Belgium
- However, SSF is not intended to provide early access or to cover off-label indications:
  - The general policy of the health insurance is to look into in-label medicines/indications
  - The SSF policy is not to take action that goes in another direction than what the health insurance/the CRM could decide
- As the SSF evaluates each case individually, it can happen for some individual cases that the SSF intervenes to fund the off-label requests of medicines
- However, it is not the future of the SFF to be used as a systematic pathway to cover demands for off-label indications

Process is cumbersome with no or unpredictable outcome→ physicians request pharmaceutical companies to provide for off-label treatments which places the decision and responsibility on the companies...
→ Arbeidsrechtbank ….

Donation of free samples
Strict conditions

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Mogen farmaceutische ondernemingen stalen verstrekken aan artsen?

★ Ja, zodat de arts een dringende omstandigheid of een medische of sociale nood kan verhelpen

Schriftelijk verzoek voor een stalen

Wat de wet zegt

Toegestaan, onder bepaalde voorwaarden:

- Alleen aan een arts
- Naar aanleiding van een schriftelijk, gedagtekend en ondertekend verzoek
- Alleen voor producten die gecommercialiseerd zijn op de Belgische markt
- Interne controle- en traceerbaarheidssysteem

Maximaal 8 stalen van hetzelfde geneesmiddel per jaar en per arts
(kleinste doos)

Free Samples
Yes, but only if commercially available

https://pharma.be/sites/default/files/2021-08/health_ethics_nl.pdf

pharma.be ALGEMENE VERENIGING VAN DE GENEESMIDDELENINDUSTRIE

Pharma.Be (association for the innovative bio-pharmaceutical industry): constructive approach, listens to the challenges encountered, shared these current opportunities with recent kick-off (working group that co-creates a potential new and sustainable framework for off-label individual medical need to better support our patients)

Reimbursement groundbreaking drugs should be faster

- **New procedure** for groundbreaking medicine
- Once EMA approval
- CTG evaluates immediate reimbursement
- No delay till reimbursement → **up to 10 months faster access**
- CTG continues evaluation
- Company pays money back if negative advice at the end of process

What about CTG? Should we re-think? More quality and faster evaluations?

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Opinion ‘working group oncology’ RIZIV/INAMI
What happens with opinion?

- Avoid parallel work: Use European evaluations in CTG/CRM
- Involve patient organisations to join evaluations
  - **Patient experts** geven perspectief van patiënten op impact van ziekte en ervaring met behandelingen (schriftelijk en/of hoorzitting)
  - **Patient organisations** (CRM/CTG)
- Involve scientific organisations to join evaluations, to help external experts and academici in CRM/CTG

Task Force BSMO: Surrogate Endpoints
Immaturity of OS-data/ clinical relevance
Fast Approval as by FDA: Data Collection Refund
Now other evidence than large Phase III RCT

Breast cancer 1

Advances in the treatment of advanced oestrogen-receptor-positive breast cancer

Nicholas C Turner, Patrick Neven, Sibylle Loibl, Fabrice Andre

www.thelancet.com Vol 389 June 17, 2017

…In a study of 50 pts with PIK3CA-mutant metastatic breast cancer, alpelisib (also known as BYL179) combined with fulvestrant was associated with a 24% objective response rate. (NCT02437318) Mayer I. et al CCR 2017

PIK3CA-mutant MBC

Our 1st Alpelisib experience in UZ-Leuven (2017)

Our Alpelisib experience in UZ-Leuven (2017)

Cancer Therapy: Clinical

Clinical Cancer Research

A Phase Ib Study of Alpelisib (BYL719), a PI3Kα-Specific Inhibitor, with Letrozole in ER$^+$/HER2$^-$ Metastatic Breast Cancer

Ingrid A. Mayer[1], Vandana G. Abramson[1], Luigi Formisano[1], Justin M. Balko[1], Mónica V. Estrada[1], Melinda E. Sanders[1], Dejan Juric[2], David Solit[3], Michael F. Berger[3], Helen H. Won[3], Yisheng Li[4], Lewis C. Cantley[5], Eric Winer[6], and Carlos L. Arteaga[1]

Clin Cancer Res; 23(1) January 1, 2017

AACR

Gene	Mutation	% reads	Class*
PIK3CA (exon 10)	c.1636C>A (p.(Gln546Lys))	28.0	class 3A
PIK3CA (exon 21)	c.3012G>T (p.(Met1004Ile))	31.0	class 3B

Clinical Benefit and improved ECOG status→ 7m later; *PTEN*m

Gen	Mutatie	% reads	Klasse*
PIK3CA (exon 10, coding exon 9)	c.1636C>A (p.(Gln546Lys))	24.0	klasse II
PTEN (exon 2)	c.105G>C (p.(Met35Ile))	26.0	klasse III
PTEN (exon 4)	c.217G>A (p.(Glu73Lys))	14.0	klasse III
PTEN (exon 5)	c.402G>T (p.(Met134Ile))	20.0	klasse III
PTEN (exon 6)	c.511C>T (p.(Gln171*))	22.0	klasse III
PIK3CA (exon 21, coding exon 20)	c.3012G>T (p.(Met1004Ile))	20.0	klasse III

7m later; *PTEN*m

5-3-2017: Dear Prof Mayer & Arteaga. 50yrs, metastatic ILC, ER+ HER-2 neg relapsed after 6L anti-E and CT. NGS (paclitaxel relapsed tumor) in Sr MJ nodule, using the Illumina-platform (targeted re-sequencing Illumina kit TruSightTumor26 Sequencing; detection limit: 5% mutante allele; 10% tumor cell)

Hoste, G et al. *Clin Drug Investig* **38**, 1071–1075 (2018).

2017: FAGG : Requirements for **Compassionate Use Medication in Urgent Situations** described in the FAGG guidance on **compassionate use** and **medical need programs** dd 04/JAN/2016. In order to ascertain that all requirements are met, you need confirmation that:

The patient is in immediate risk of dying;

- Or the risk of non-treatment is higher than the inherent risks of the treatment
- The patient cannot be treated
  - With a marketed medicinal product,
  - With a product under hospital exemption or
  - With a magisterial preparation
  - In a clinical trial

1. treatment plan protocol
2. informed consent form (ICF; no name)
3. CUP attestation form
4. template to notify hoofdarts & EC
5. Letter of agreement with UZ Leuven
5. AE-SAE formulier: <24h SAE; <10d AE
6. Duration to get drug on average 3m…

‘ I am responsible for the use of
- Unauthorized drug
- Authorized drug in unauthorized indication’

I. Global CU program = UMN Belgium
1 Individual patient

Novartis Global Medical Affairs→ Individual UMN
Alpelisib (BYL719) “CBYL719X2001”

Opened 17-1-2017

II. Belgian CU program
2nd patient …
Project KOTK Laurence
UZL + UZ Gent

Novartis Pharmaceuticals
BYL719 Compassionate Use Program
Patient Request Form

BYL719_CUP_Patient Request Form
Version 0_February 2018_Belgium

Product: BYL719

Indication: Alpelisib (BYL719) in combination with fulvestrant or letrozole for postmenopausal women and men with endocrine resistant hormone receptor-positive (HR+) HER2-negative (HER2-) metastatic breast cancer, who have recurrence or progressed after at least 3 lines of systemic treatment for advanced or metastatic disease, and who harbor specific PIK3CA hotspot mutations.

CPO contact information:
Lynn Vandamme, Medical Advisor Oncology, Medialaan 40 bus 1, 1800 Vilvoorde, Belgium.

Please scan the completed form and an email to belgium.oncology@novartis.com. Novartis will assign a global patient number once all criteria are met. No exceptions to Eligibility Criteria are permitted.

In accordance with article 106 §5 of the Royal Decree relative to the human and veterinary medicines as modified on 25 April 2014, I confirm that the FAMHP does not have any objection to the demand for modification to the here above mentioned compassionate use program

8-3-2019: BSMO meeting (Task Force Breast) PIK3CA testing is required in all luminal HER2-neg MBC patienten (30 % +)

famhp
federal agency for medicines and health products
10 years
Your medicines and health products, our concern

Federal agency for medicines and health products
Eurostation II - Place Victor Horta 40/40
1060 BRUSSELS
www.afmps.be

III. Belgian CUP/MNP 12-2020

Onderwerp	Goedkeuring van een wijziging van een programma voor gebruik in schrijnende gevallen op 22/12/2020
Titre de l'objet	Approbation d'une modification d'un programme d'usage compassionnel le 22/12/2020
Subject	Authorisation of a modification to a compassionate use program dated 22/12/2020

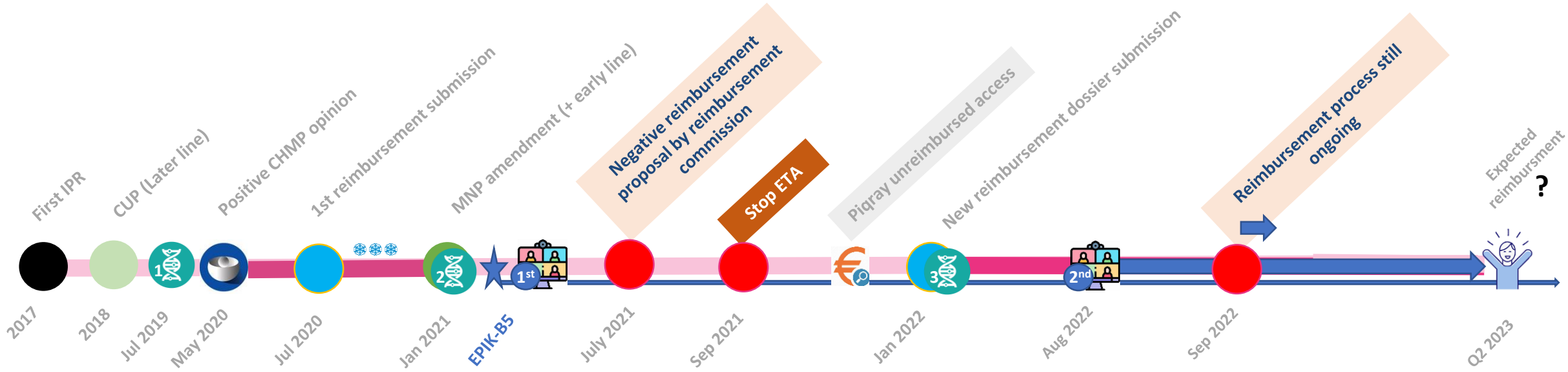
Medicinal product : alpelisib (BYL719) (50mg and 200mg, tablet)

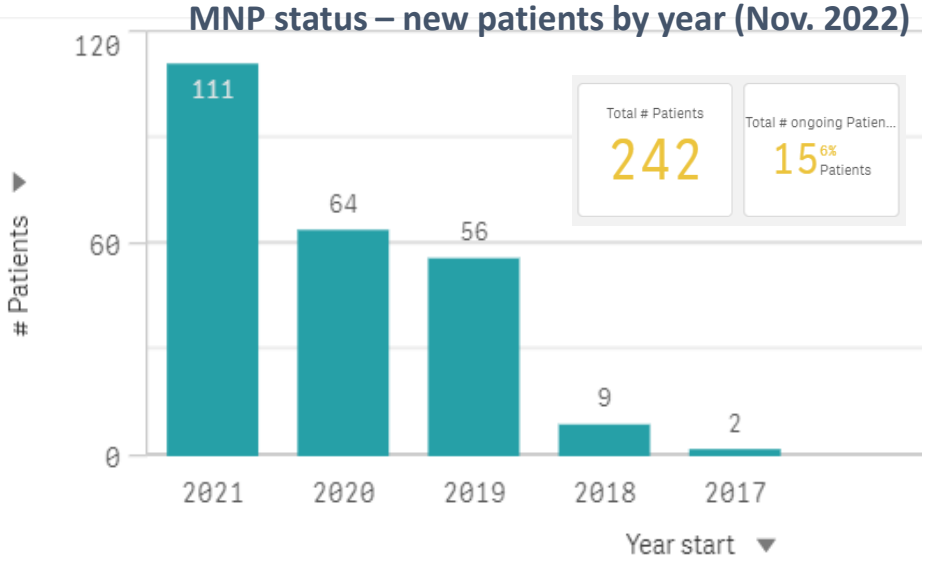
Indication : in combination with fulvestrant or letrozole for men and postmenopausal women with hormone receptor-positive (HR+) HER2-negative (HER2-) advanced breast cancer, who have recurred or progressed on or after an endocrine-based treatment, and who harbor a PIK3CA mutation

Modification: alignment with EMA approval + extension to all activating PIK3CA mutations

Ethics Committee designated: UZ Leuven
Reference: CUP-201723a2

Piqray access journey in Belgium[1,2,3]





Acces to Piqray today: ★ EPIK-B5 (ph III: ALP+FUL vs FUL; post CDKi; HR+ HER2- aBC) – 6 centers in Belgium

Reasons for first negative reimbursement decision:

- No effect on OS; ↑↑ Drop-out due to AEs
- Label not in line with current guidelines; lack of comparison vs. everolimus+exemestane/chemo

- EU reimbursement status: Even NICE reimburses

[1]Alpelisib | FAGG; [2]Novartis – aanvraagdossier voor terugbetaling Piqray^{O} (2020) en evaluatierapporten (CTG); [3]Novartis – aanvraagdossier voor terugbetaling Piqray^{O} (2021) en evaluatierapporten (CTG)
Piqray Product Information: SmPC (=LINK)

1 PIK3CA NGS Testing for BC if associated therapy reimbursed
2 PIK3CA NGS Testing for BC
3 PIK3CA NGS BC Tumoral Tissue & Liquid biopsy
Hearing with KOLs
RIZIV/INAMI Covid freeze

Reimbursement status in EU countries & UK

➔ Piqray has been reimbursed in 14 countries in EU including UK[2,3,4,5,6]

4,5

NICE National Institute for Health and Care Excellence

Final appraisal document

Alpelisib with fulvestrant for treating hormone receptor-positive, HER2-negative, PIK3CA-mutated advanced breast cancer

1 Recommendations

1.1 Alpelisib plus fulvestrant is recommended as an option for treating hormone receptor-positive, HER2-negative, PIK3CA-mutated, locally advanced or metastatic breast cancer in adults, only if:

- their cancer has progressed after a CDK4/6 inhibitor plus an aromatase inhibitor and
- the company provides alpelisib according to the commercial arrangement (see section 2).

6

Positive decisions to reimburse alpelisib in:

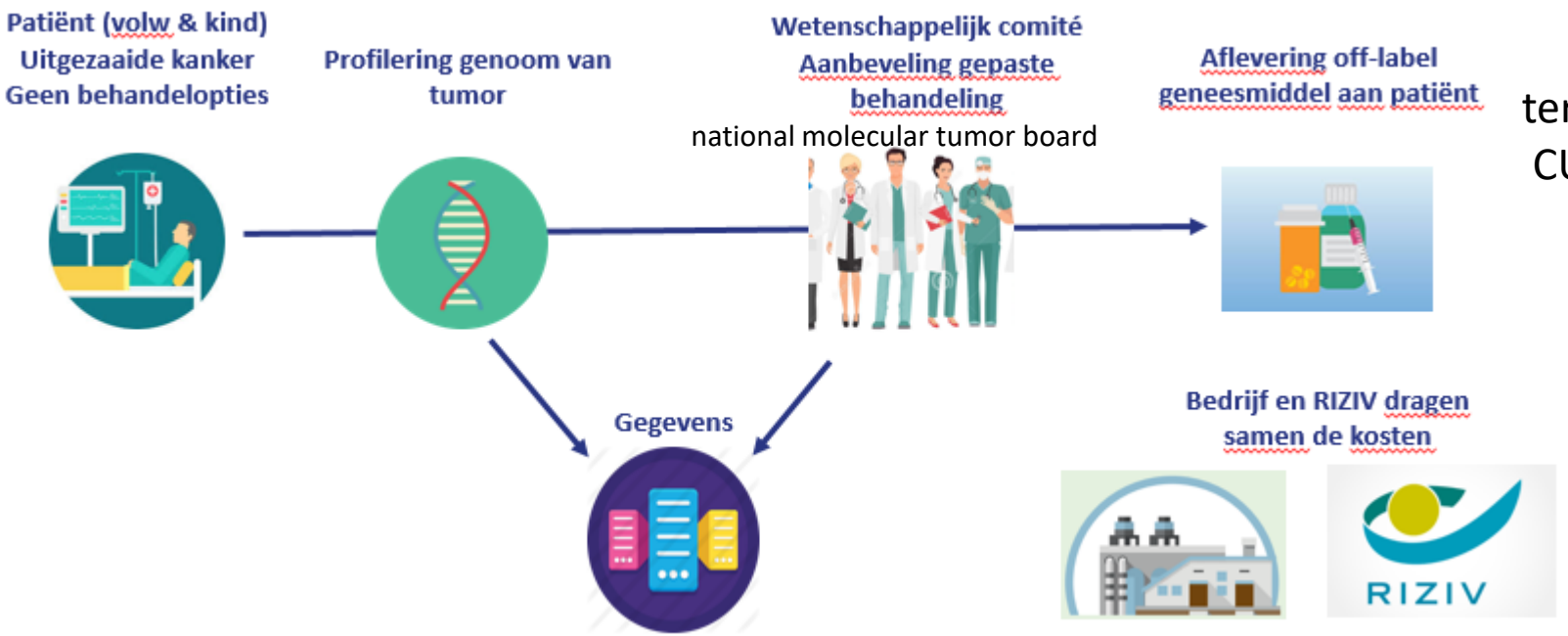
The Netherlands
Austria
Switzerland
Luxembourg
Sweden
Slovenia
Italy
Spain
Croatia
Finland
Norway
Iceland

[2]Novartis – aanvraagdossier voor terugbetaling Piqray^{O} (2020) en evaluatierapporten (CTG); [3]Novartis – aanvraagdossier voor terugbetaling Piqray^{O} (2021) en evaluatierapporten (CTG); [4]https://www.nice.org.uk; [5]https://www.novartis.com/uk-en; [6]Governmental Healthcare portal from each respective country; Piqray Product Information: SmPC (=LINK)

No Access:
Neratinib Metastatic

Neratinib after 3L in monotherapy or in combination with capecitabine is a useful treatment for patients with and without brain metastases. PFS and OS were found to be similar as previous trial data. Routine anti-diarrhoeal prophylaxis allows this combination to be safely delivered to patients in a real-world setting. 45 patients from Royal Marsden BCRT 2022 October

No legal framework in B. for Individual off-label use of medicinal products
Pierre Fabre…as it is available in Germany and UK…try to obtain it from there
No intention to start a marketing authorization application process in B.



terugbetaald, klinische studie, CUP/MNP, nieuw systeem

- Procedure should be faster; no layer of administration
- Budget for off-label molecular guided treatment if EB-recommendation by an independent body?
- National Molecular Tumor Board
- Decision to administer/prescribe ~doctor (supported by nMTB, MOC) national EC
- Collection of real-world data: to learn from and to support drug for reimbursement

Ballett study (P.I. Dr. Brigitte Maes)

- **B**elgian **A**pproach for **L**ocal **L**aboratory **E**xtensive **T**umor **T**esting
- 936 Belgian pts with metastatic solid tumors

Comprehensive Genomic Profiling report +/- Treatment recommendation

Informed consent
- Ballett (13 sites)
- Precision 1

Data collection
Precision 1

CGP
TruSightOncology 500
9 Belgian laboratories

Molecular Tumor
Board
Dedicated Platform

Reimbursed Rx
Clinical trial
MNP/CUP
Off-label Rx

illumina®

OncoDNA
THE CANCER THERANOSTIC COMPANY

pieriandx

JESSA ZIEKENHUIS

BSMO

What is going to be done by Health Authorities

DOC 55 **2934/012**

CHAMBRE DES REPRÉSENTANTS
DE BELGIQUE

28 octobre 2022

NOTE DE POLITIQUE GÉNÉRALE (*)

Santé publique

DOC 55 **2934/012**

BELGISCHE KAMER VAN
VOLKSVERTEGENWOORDIGERS

28 oktober 2022

ALGEMENE BELEIDSNOTA (*)

Volksgezondheid

Early and fast access is important issue for our Minister of Health

Taskforce KCE: Report 348, 2022 who are these pts?
→roadmap for development, production, mark authorization, reimbursement if clinical benefit

RIZIV workgroup: Contractgeneesmiddelen; modernisering terugbetalingsprocesen

Internationalising of reimbursement: treatment combinations, complexe therapies
eGezondheid 2022-2024

PASKWIL

Thank you
for your attention

Access of Belgian cancer patients to therapeutic innovations