PHARMA SUMMIT 24

Exploring Values of Healthcare

18th April 2024

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Speaker Slides:

Neil Grubert, Market Access Consultant

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Neil Grubert,

Global Market Access Consultant

Neil Grubert Consulting

"Market Access Trends in Europe"

Bio:

Neil is a pharmaceutical market access specialist with more than 20 years of experience tracking access environments around the world. He advises leading biopharmaceutical companies on the implications of international access trends and has authored more than 150 reports on the subject. Neil also delivers multi-client and bespoke training programmes with a focus on pricing, reimbursement and market access trends around the world.

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Market access trends in Europe

Neil Grubert

18th April 2024

Neil Grubert Consulting

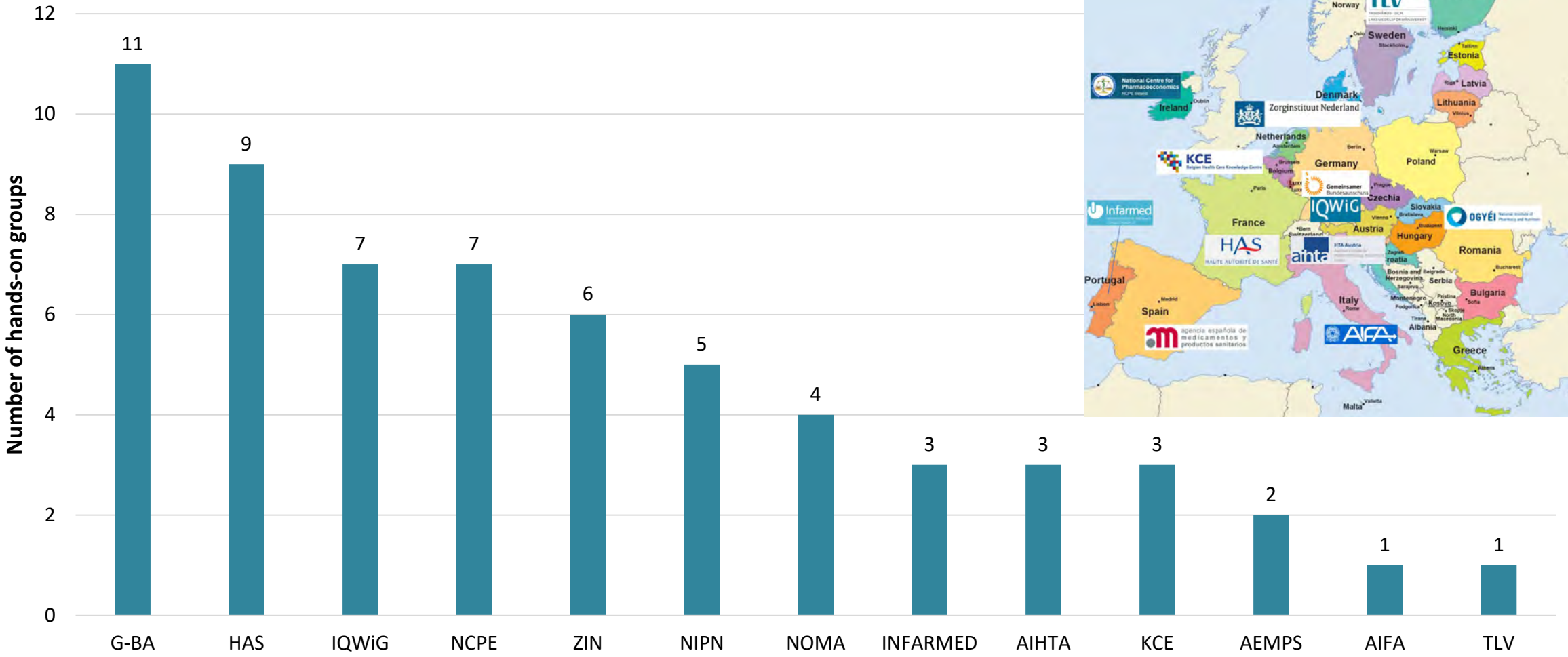
pmi PHARMA SUMMIT 24
Exploring Values of Healthcare

Agenda

- EU joint HTA
- Revision of the EU general pharmaceutical legislation
- Cross-border access collaborations
- Key HTA developments in the big 5 European markets
- Managed entry and innovative access arrangements
- Key lessons from other European countries
- Governments are looking to boost domestic drug development and production
- Outlook: evolution of launch environments
- What do you need to do to prepare for the future?

Agency representation in hands-on groups that have shaped pan-European HTA

- 13 agencies were represented in EUnetHTA 21, which has shaped pan-European HTA.
- Germany had the strongest influence through the G-BA and IQWiG.



First implementing act for EU joint HTA

- Confirmation of timelines in parallel with marketing authorisation process.
- JCA Subgroup should aim for the lowest possible number of PICOs.
- Potential for some involvement of manufacturers in assessment scoping “if necessary.”
- Stakeholders with expertise covering several Member States will be prioritised for consultation.
- Company’s JCA dossier must include details of any “profound” epidemiological differences between countries, information on substantial variations in clinical pathways, details of early access/compassionate use programmes and HTA reports from EEA states, Australia, Canada, the UK and the US.

National HTA agencies' perceived readiness for EU joint HTA

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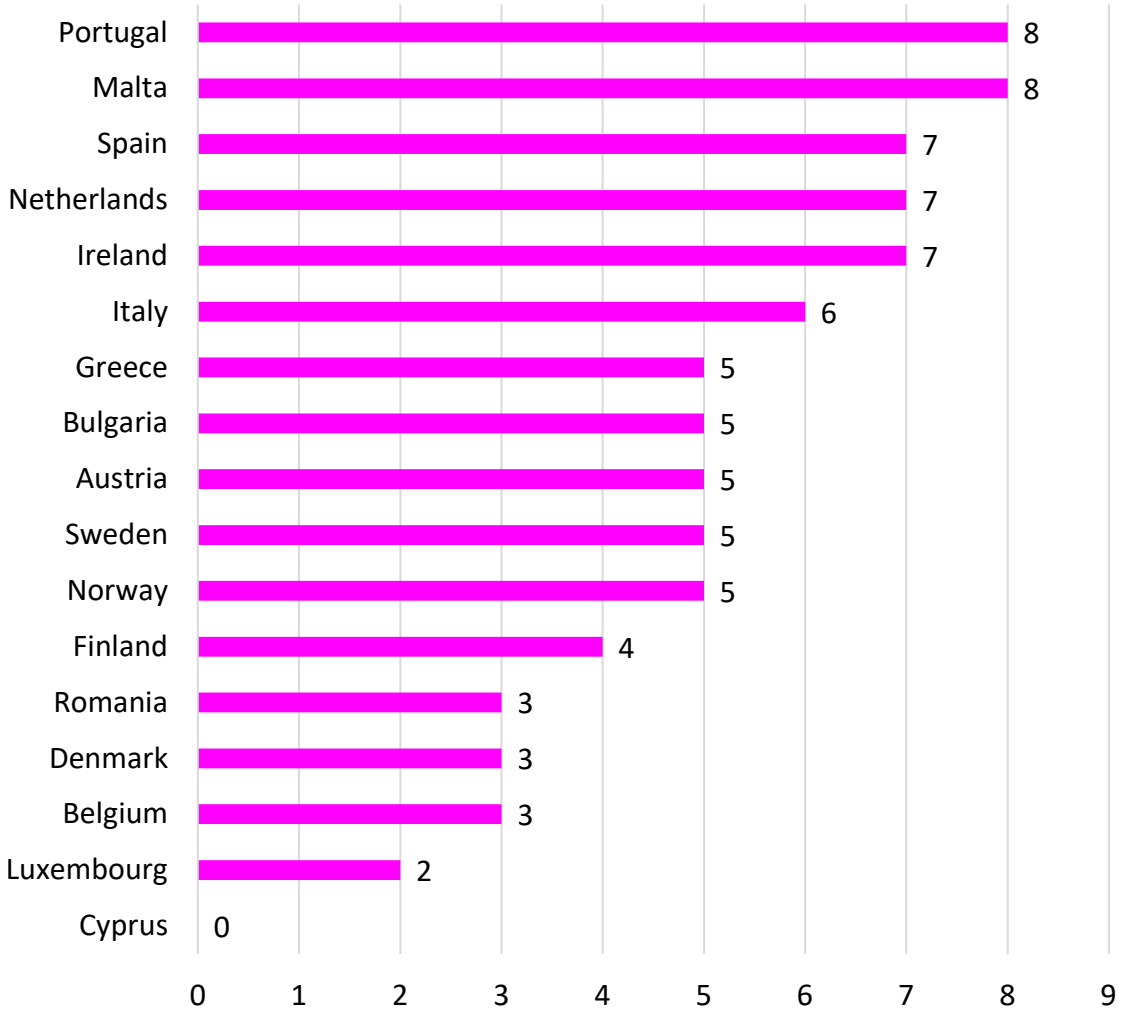
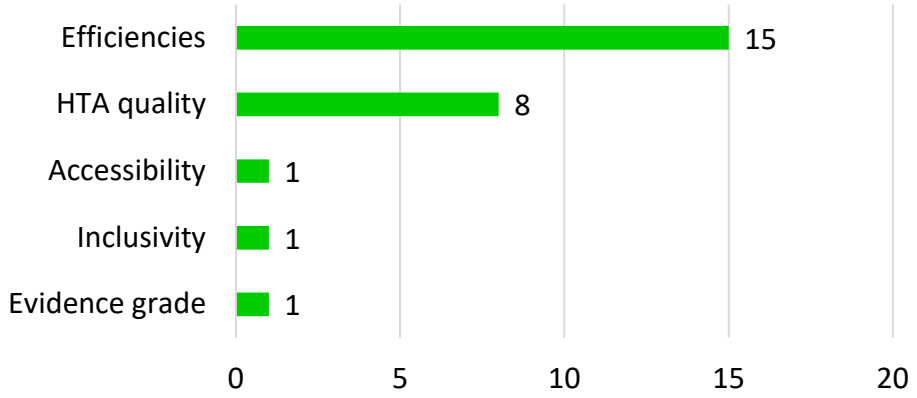
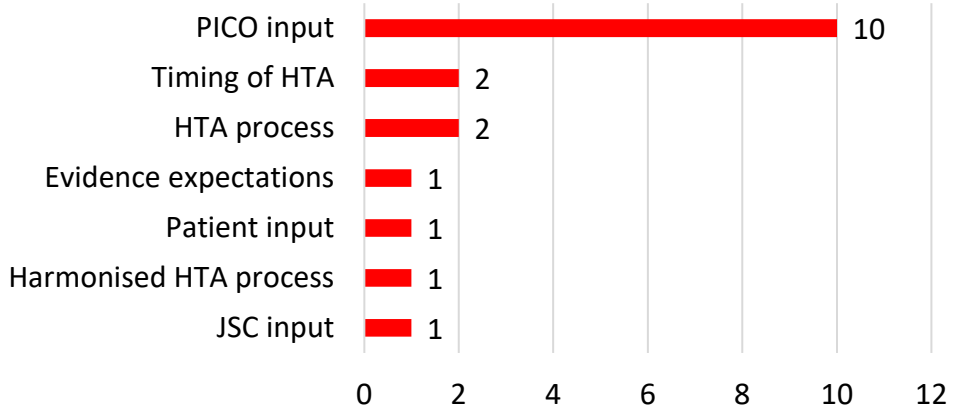
HTA agencies' assessment of their readiness



Key opportunities of EU joint HTA



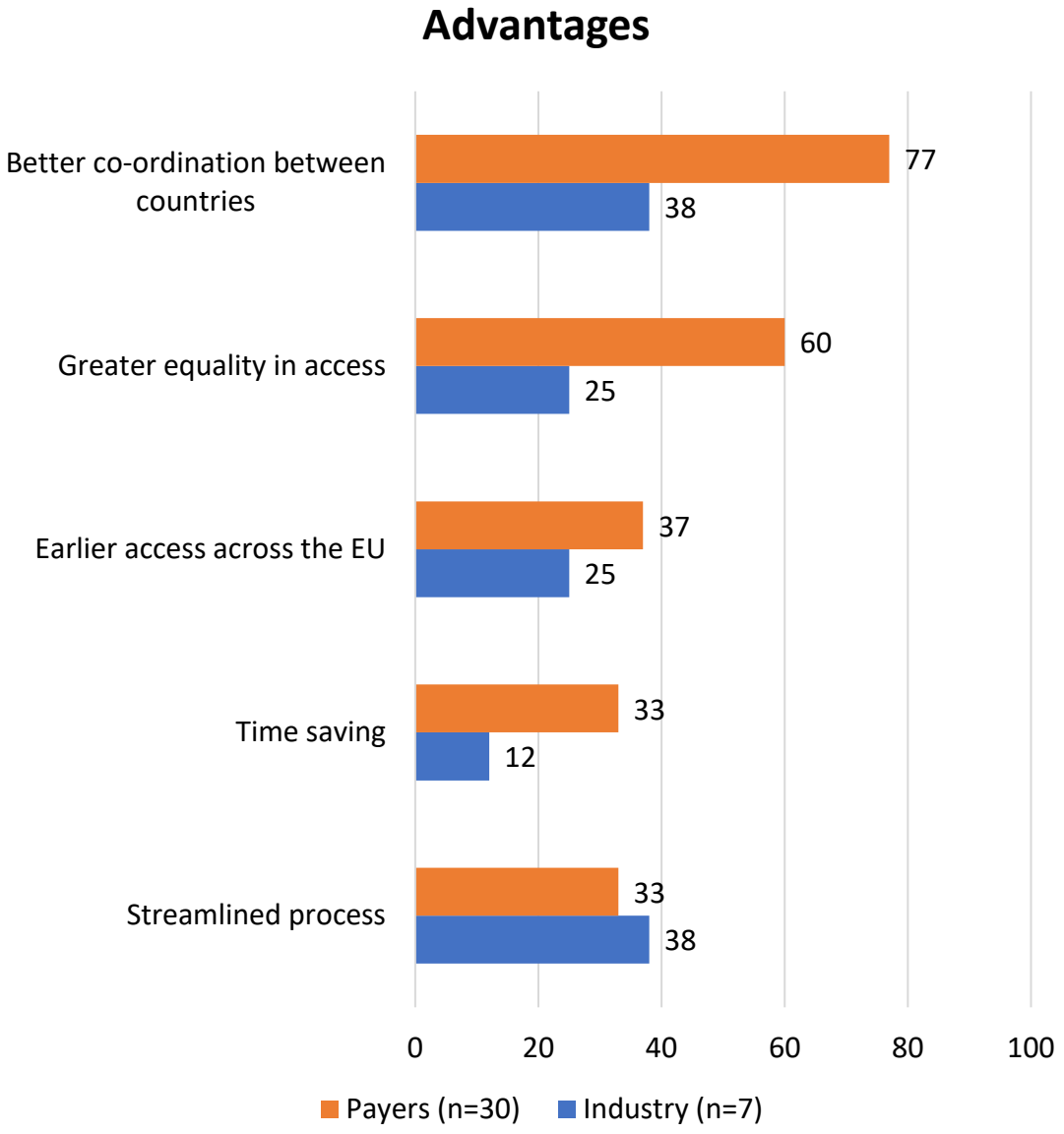
Key challenges of EU joint HTA

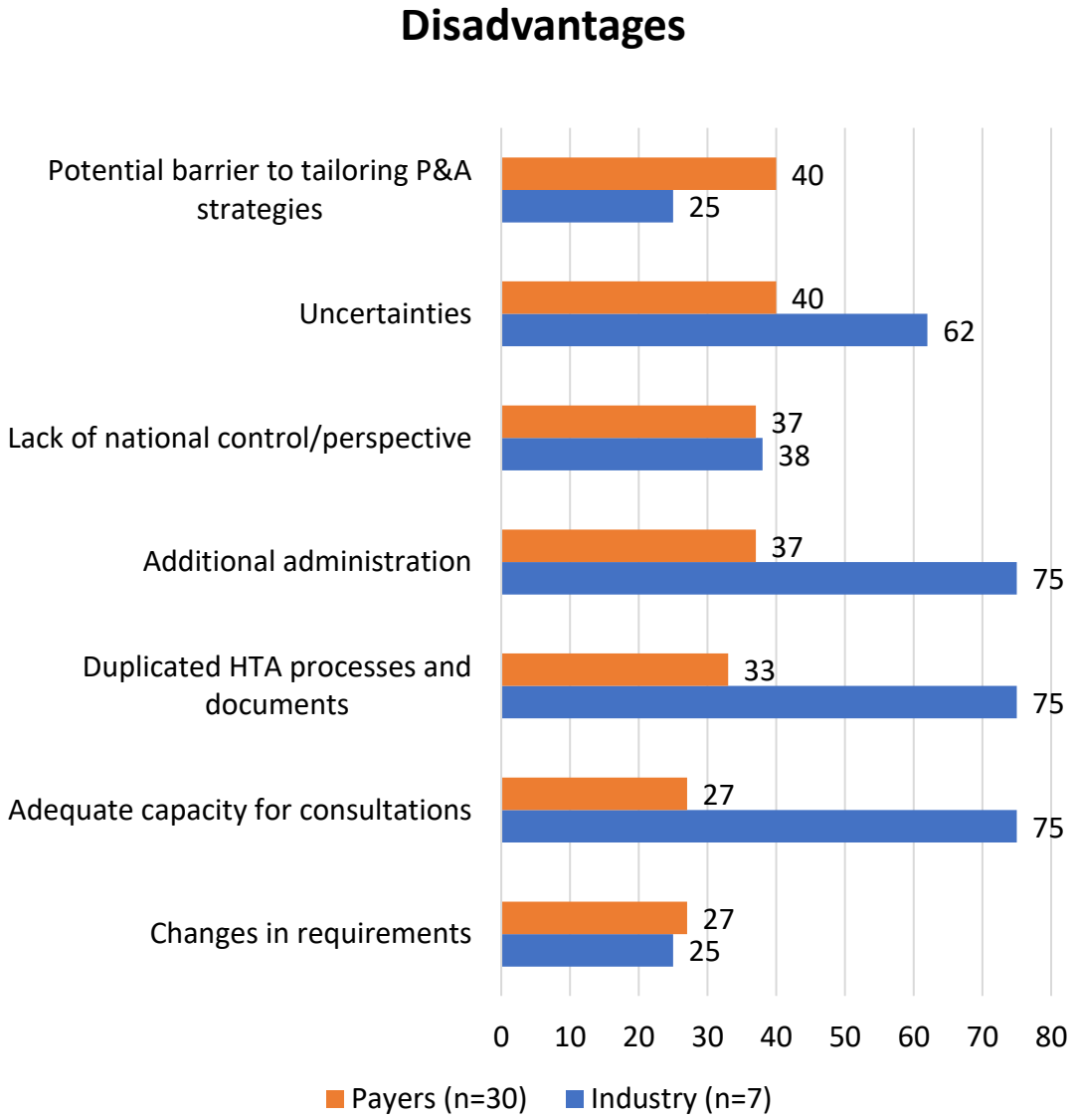


How does pharma assess readiness for EU joint HTA?

- Dr Vanessa Schaub, Head of Global/EU HTA Strategy at Roche, is concerned by “significant gaps in readiness.”
- Roche is adapting existing resources, establishing cross-functional and specialist teams and working to anticipate EU-level PICOs.
- Adequate resourcing of the European Commission’s Secretariat and the Coordination Group is not yet in place.
- Industry is concerned about “the lack of transparency and detail available about the JCA procedures and methodologies at this advanced stage.”
- Joint HTA has the potential to be “a meaningful contributor to faster access” but could become “an additional hurdle and extra administrative process.”
- Schaub warns that “an experimental approach will jeopardise success before it has even commenced and must be avoided if we are to enable timely, high-quality and trusted EU HTA outputs right from the start.”

Differing payer and manufacturer views of JCA





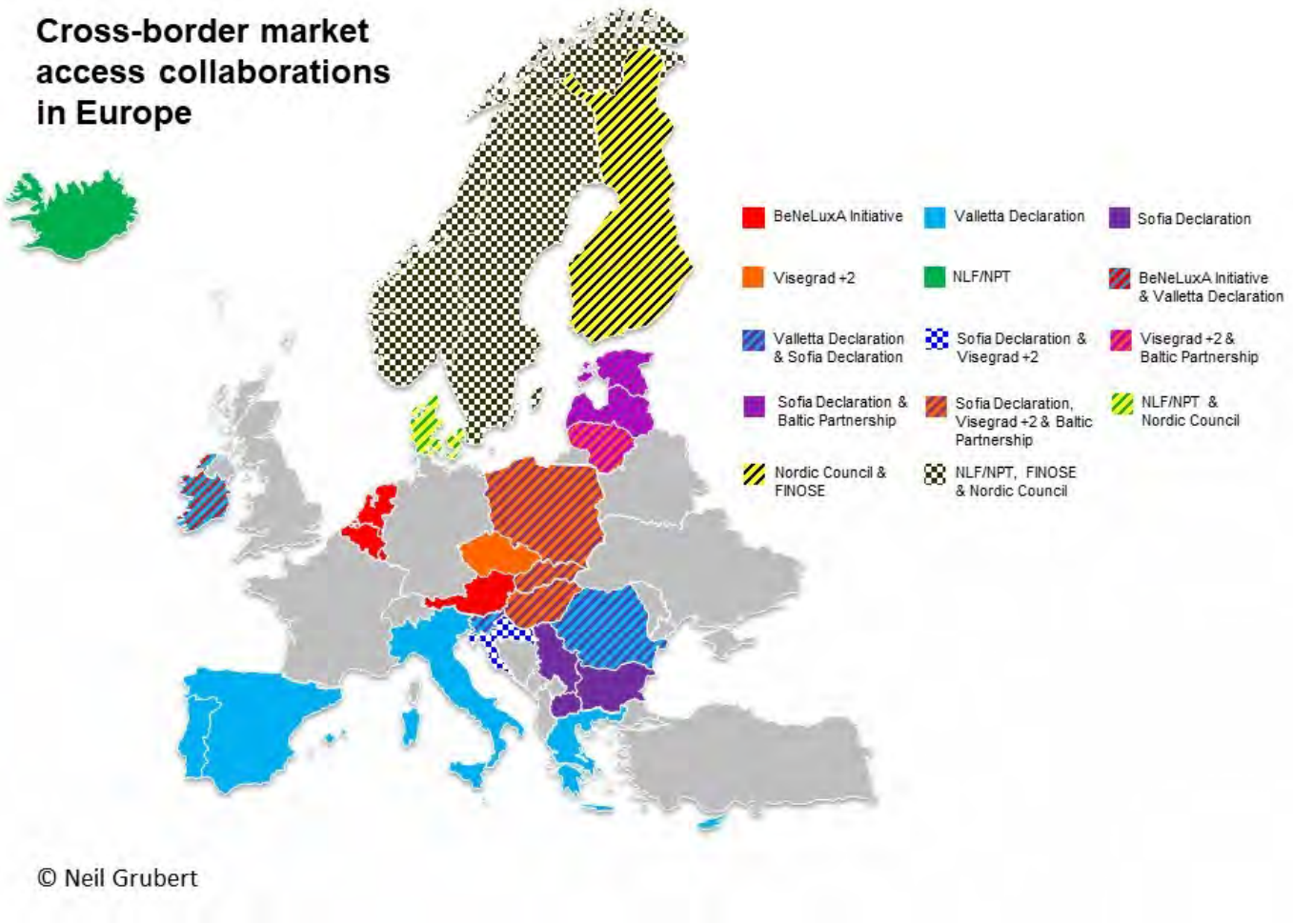
Source: “EU Joint Clinical Assessments On Health Technologies Is Approaching—Are We Prepared?”, IPSOS

EU pharma reform clears a crucial hurdle

MEPs have approved revisions to the Commission's proposals:

- Baseline regulatory data protection period would be 7.5 years, with up to one additional year for meeting certain conditions.
- Market protection would be two years, plus an extra year for securing approval for a significant new indication.
- Orphan market exclusivity would be 9 years, with a two-year extension for addressing high unmet medical need.
- Commission would facilitate sharing of P&R best practice, issue guidance on MEAT criteria in tendering and promote the activity of NCAPR.
- Requirements for timely launch would have concessions for orphan drugs and ATMPs, a list of exempt products and a conciliation mechanism.
- New EU Access to Medicines Notification System could be expanded to broader areas of pricing within 5 years.
- Comparative clinical trials would be encouraged.
- Cross-border exchange of ATMPs prepared under hospital exemption could be authorised in cases of medical need.

Expansion of cross-border collaboration activity in Europe



- Beneluxa Initiative has focused largely on ATMPs.
- FINOSE has been enlarged by the accession of the Danish Medicines Council and is increasingly working with the Nordic Pharmaceutical Forum.
- National Competent Authorities on Pricing and Reimbursement (NCAPR) has European Commission backing and EU4Health funding.
- Project EURIPID is now a regular member of the NCAPR.
- Novel Medicines Platform is a multi-stakeholder platform involving 53 countries that seeks “solutions to improve access to effective, novel and high-priced medicines.”
- SUSTAIN-HTA will support the HTA Coordination Group and its Subgroup on Methodology and “aims to assist in the alignment of HTA methodologies.”
- Eight countries have expressed interest in voluntary cooperation on joint HTA.

Expansion of cross-border collaboration activity beyond Europe

Name	Participants	Key objectives
Project Orbis	Australia, Brazil, Canada, Israel, Singapore, Switzerland, UK, US	Accelerated regulatory approval of cancer therapies
Access Consortium	Australia, Canada, Singapore, Switzerland, UK	Accelerated regulatory approval of non-oncology drugs
UK Int'l Recognition Framework	UK, Australia, Canada, EU, Japan, Switzerland, Singapore and US*	UK recognition and lighter-touch evaluation of marketing authorisations of cutting-edge drugs from 7 other regulatory authorities
AUS-CAN-NZ-UK Collaboration	Australia, Canada, New Zealand, UK	8 "like-minded" HTA bodies will share information on best practice and conduct a pilot JCA
-	Canada, Belgium, Denmark, Iceland, Ireland, Netherlands, Norway, Portugal, Sweden	Canada will work with members of Beneluxa Initiative, Nordic Pharmaceutical Forum and Portugal to share experiences on dealing with lack of evidence and negotiating prices for high-priced drugs

* UK MHRA recognises decisions of other designated regulatory agencies

- Project Orbis approved 10 new cancer drugs and 8 new oncology indications from May 2021 to December 2023 and has shown "exceptional worth in clinical situations where time really does matter."
- Access Consortium "creates a market of some 160 million people, which is attractive to developers."
- UK's new International Recognition Procedure will be "a very important tool in our regulatory toolbox": first drug—Amgen's Xgeva—was approved within 30 days.
- AUS-CAN-NZ-UK Collaboration Arrangement will conduct at least one pilot joint clinical assessment.
- NICE International is increasingly active, especially in APAC and Latin America (including a bilateral agreement with Taiwan).
- Pan-American Health Organization (PAHO) participated in a recent meeting of the Novel Medicines Platform.

Key HTA developments in the big 5 European markets

Plans to adapt to immature evidence base of many new medicines.

Interministerial mission recommends increased use of RWD and more weight for QoL and health economics.

GBA and IQWiG remain sceptical about RWD in general.

RWD data collection through registries has disappointed GBA: underestimated the effort and expense, overestimated quality of existing registries and misjudged incentive for manufacturers to collect RWD.

Health economic evaluation formally integrated into decision making in new AIFA guidelines in March 2021.

Reorganisation of AIFA intended to accelerate access, but likely impact remains unclear.

Court ruling challenged inclusion of health economic evaluation in therapeutic positioning reports (IPTs).

Legislation to reform the HTA system is expected imminently.

Health economic evaluation is expected to become the norm.

NICE reforms included new disease-severity modifier, new approaches to evidence, more flexibility in considering uncertainty, earlier engagement on commercial/managed access proposals.

New proportionate approach can cut appraisal times by 45%.

Shift towards living guidelines.

Three main objectives of managed entry

Objective	Managed entry approach	Level of activity
Control budget impact	Financially-based agreements	Population/patient
Tackle uncertainty	Coverage with evidence development	Population
Manage variable drug response rates	Outcomes-based agreements	Patient

Innovative access arrangements

Innovative contracting	Potential application
Instalment/annuity payments	One-time potentially curative therapies (e.g., gene therapies)
Warranties	High-cost drugs with variable response rates (e.g., cell and gene therapies, oncology drugs)
Subscription (“Netflix”) model	Drugs with high price and potentially large patient population (e.g., direct-acting antivirals for hepatitis C)
Portfolio pricing	Portfolio of drugs for a single indication (e.g., cystic fibrosis)
Delinked payment	Drugs that will not be routinely prescribed (e.g., reserve antibiotics)
Indication-specific pricing	Drugs used for multiple indications (e.g., oncology drugs, auto-immune therapies)
Population health management	Drugs used to prevent/manage common chronic diseases (e.g., treatments for cardiometabolic disorders)

Managed entry and innovative contracting: a mixed picture

CEPS has not been enthusiastic about OBAs, but LFSS 2023 paved the way for deals with instalment payments for ATMPs.

Some health insurance funds have negotiated OBAs, but GKV-SV has not been receptive.

Volume-based agreements will become more common.

Since the introduction of the innovative medicines funds in 2017, risk sharing has been largely superseded by appropriate prescribing registries, but new payment *at* results has been tried for three ATMPs.

VALTERMED platform has helped to boost OBAs at national level.

Catalonia is experimenting with innovative contracting.

Cancer Drugs Fund has increased use of CED in oncology, but Innovative Medicines Fund has been slow to take off.

NHS England favours "smart deals": many offer rapid, broad access in return for competitive pricing.

Orphan Drug Access Protocol pilots an unusual hybrid managed entry approach that combines patient-level outcomes-based reimbursement with population-level CED in non-oncological rare diseases with low sales.

TLV has developed a simulation tool for OBAs and believes they can address key issues related to ATMPs.

Key lessons from other European countries

Free pricing with reimbursement at launch allows immediate access pending HTA, price negotiation and retroactive application of rebates.

The focus of cost containment is shifting from launch to maturity, with increased support for early access and a new direct access pilot project.

Special fund for innovative medicines will grow by 30% in three years and Law 648/96 and AIFA National Fund provide access to promising drugs before general reimbursement.

NHS England is keen to accelerate and broaden access by negotiating "smart deals" and using coverage with evidence development to address uncertainty in promising drugs.

The TLV sees a growing role for outcomes-based agreements, flexible pricing for combination therapies and pricing linked more explicitly to prevalence.

Early access will allow simultaneous regulatory and reimbursement decision making and speed up access for some drugs by up to 3 months.

Governments are looking to boost domestic drug development and production

EU wants pharmaceutical industry to "innovate, flourish and continue to be a global leader."

Pricing policy has "damaged the industrial fabric" of the pharmaceutical sector. Ambition is to "make France the leading European nation in health innovation and sovereignty" by 2030.

A round table for the healthcare industry will seek to improve conditions for research, development and manufacturing in Germany. New legislation this year will promote pharmaceutical R&D and production in the country.

Italy wants a sizeable share of €1 trillion global pharma investment in coming years. Needs to create an access environment more conducive to innovation.

Pharma is a "key player" in economic reconstruction because of "its productive capacity" and "the traction it exerts on other sectors."

Life Sciences Vision aims to "make the UK the best place in the world to discover, develop, test, trial, launch and adopt new treatments and technologies."

Outlook: evolution of launch environments

Future launch environments will be characterised by more complex patient journeys, higher evidence requirements, greater uncertainty and shifts in care settings

	2010-2013	2014-2019	Future (without pandemic)	Post-pandemic future
Stakeholder environment	Simple: payers then prescribers	Complex: web of stakeholders—payer-led	Personalised and patient journey based	Increased patient journey complexity
Payer priorities	Price	Value-based, HTA	Outcomes, RWE, new funding approaches	Tighter budgets, higher evidence bar
Launch positioning	First line, mass market	Later line, segmentation	Companion diagnostic, biomarker, genotype	Complex, uncertain environment
Launch type	Small molecules and biologics increasingly specialty	High-cost specialty, orphan drugs, ATMPs	Further diversification, digital therapeutics, diagnostics	Self-/home-administered drugs have new advantage

Source: Adapted from *IQVIA Launch Excellence VII*

What do you need to do to prepare for the future?

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- Be aware of the growing divergence between regulatory and access requirements.
- Follow the work of relevant cross-border collaborations.
- Explore opportunities to work in partnership with healthcare systems.
- Keep up with changes in HTA processes—national and supranational.
- Be prepared for increasing pressure for transparency (with IRP repercussions) and cost-plus pricing.
- Recognise the potential—and limitations—of managed entry and innovative contracting.
- Plan ahead for changes in cost containment across the life cycle.
- Understand how governments will seek to boost local drug development and production: carrots or sticks?
- Anticipate how the relative attractiveness of different markets may change significantly.
- Understand how health policy, economic policy, industrial policy and geopolitics will shape global pharma market—and disseminate that knowledge in your organisation.

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Outlook for access in Spain

- Royal Decree on HTA will be published before the summer: therapeutic positioning reports and health economic evaluation may not be needed for reimbursement of every drug
- VALTERMED is not comparable with RCT data and can become

What do HTA agencies want from real-world evidence?

- Real-world data (RWD) ofte
  and providing reassurance f
- In some cases—for example
  data source—committees m
- For NICE, key considerations

JCA simulation in oncology resulted in up to 57 PICOs

- Simulation of joint clinical assessment for three recent cancer drugs resulted in 16, 22 and 57 PICOs
- Consolidation reduced these numbers to 7, 6 and 23 PICOs
- All three technologies would require use of indirect treatment comparisons or network meta-analyses
- RWE informed the comparative effectiveness analysis in two of the products, signalling the importance of observational data
- All scenarios would present challenges in meeting EUnetHTA 21 proposals for data acceptability and comparisons

Could platform trials be required by NHS England for some new drug classes?

- Board meeting discussed preparations for up to 28 late-stage disease-modifying dementia therapies
- Members noted "the modest clinical benefits, safety concerns and intensiveness of the treatment regime," as well as a potential NHS bill of £500mn to £1bn
- Pilot projects to prepare for the launch of new drug classes with a potentially substantial budget impact are already taking place with anti-obesity medications

SUSTAIN-HTA will seek to "bring everyone on board" in using new HTA methods

- The trend towards personalised medicine and smaller patient populations requires new approaches to HTA

What are pharma's main concerns about AMNOG and cost containment in Germany?

Olaf Weppner, Managing Director of AbbVie Deutschland, highlighted several key issues in a recent article:

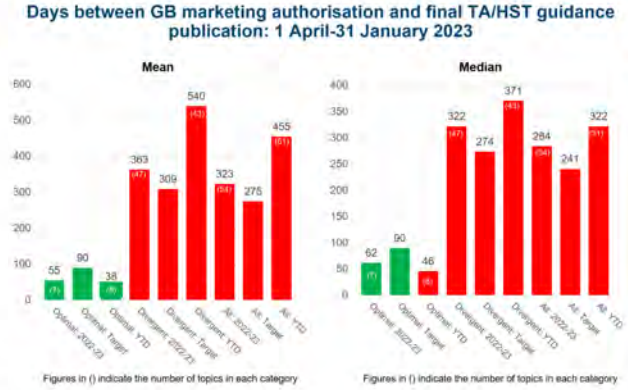
- He believes "AMNOG must becon
  overall"
- Recent cost-containment measur
  complex and, because they are a
  "absurd total discounts"
- Lack of recognition for incremental
  further concern
- He warns that "poorer reimbursem
  that global companies are looking

Outlook for AIFA and managed entry in Italy

"We absolutely must … return to managed entry agreements that can enhance the value of the product, allowing the country to deal with expenditure with a different reasoning. Cutting the price of a drug by demanding a very high discount means, on the one hand penalising the manufacturer, which in all events must have an economic return, but above all demonising the treatment in question. Because the drug or cell or gene therapy deserves attention from our healthcare system as a potential source of savings. … If AIFA fails to recognise the value of certain treatments in the future, companies could also minimise the value of the Italian market in the global context."

Are access delays in the Netherlands levelling off?

How is NICE doing on timelines?



	RAG
Optimal	Green
Divergent	Red
All	Red

Commentary

- High confidence that the targets will be achieved for optimal medicine publications, as YTD performance is significantly lower than target. Already published more optimal topics YTD than the 22-23 year.
- Low confidence that mean and median targets for divergent and all topics categories can be achieved.
- TA publications (45) have a mean of 305 days, however HST publications (6) have a mean of 1,625 days which is significantly affecting the divergent and overall data for 23-24.

NICE

"An unworkable framework" for JCAs

EFPIA, EUCOPE, EuropaBio, the Alliance for Regenerative Medicine and Vaccines for Europe have published a joint position statement on the first implementing act on joint clinical assessment

- Associations criticise the lack of involvement of manufacturers and unrealistic timelines
- Member States would have 140 days to develop PICOs, compared with 90 days for companies to prepare their submission dossiers
- SMEs would be particularly disadvantaged by the lack of opportunity for early engagement with assessors
- JCA process would be better served by allowing companies to submit all relevant information
- Earlier involvement of companies through a scoping meeting would make the scoping phase more efficient
- By helping companies to anticipate different PICO scenarios, the quality of the submission dossier and JCA reports would be enhanced
- Companies could confirm their intention to file for a marketing authorisation in advance, enabling HTA authorities to start their preparations earlier

EU working on joint procurement of orphan drugs through DG HERA

- Czech Deputy Minister of Health, Jakub Dvořáček, commented on cross-border collaboration at a recent symposium
- EU is currently working on how to initiate a DG HERA mandate for joint procurement of rare disease therapies through a loophole in legislation
- Joint procurement of monkeypox vaccine offers a useful precedent
- Funding paths could be a challenge in some countries
- Czech Republic is currently an "informal observer" at Beneluxa Initiative's meetings and could consider closer cooperation

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AXIS Healthcare Consulting were the platinum sponsor at the 2024 Pharma Summit for the second year running. Founded and led by Brenda Dooley, they are a specialist boutique HTA agency with an in-house expert team with experience in NICE, SMC and NCPE appraisal processes. AXIS's depth of extensive HTA experience make them the trusted partner for reimbursement success, offering a full suite of HTA support in UK and Ireland.
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