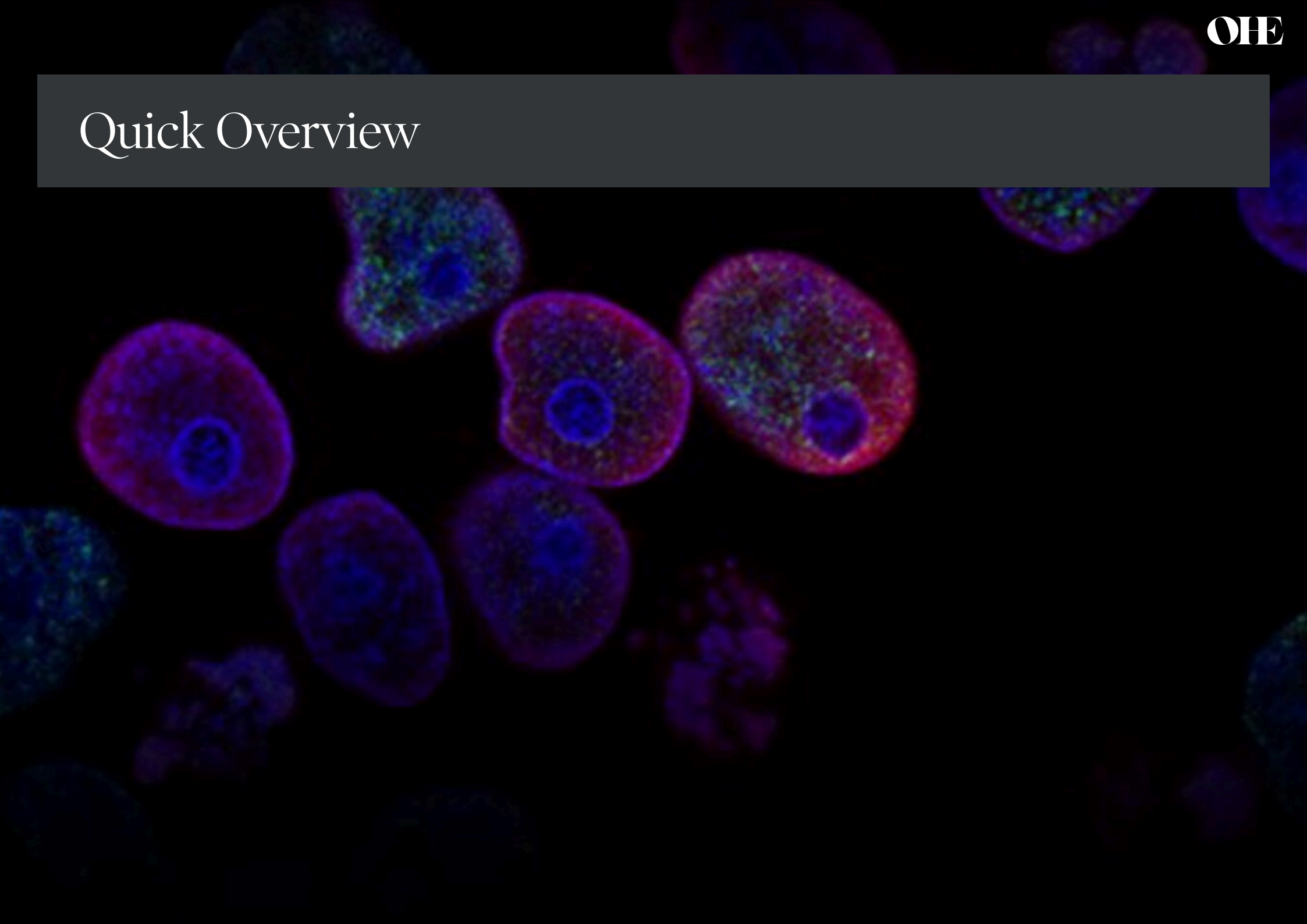
Gene therapies: are we ready?

Event summary

Quick Overview



The debate

Gene therapies: are we ready?

Gene therapies, many of which offer short-term treatment regimens, directly address the underlying cause of disease. As a result, they could bring transformative benefits to patients by halting disease progression and in some circumstances they may offer the prospect of a cure.

Questions are being raised in Europe around whether adopting these technologies into routine clinical care threatens the financial sustainability of health systems. There are also concerns regarding the challenges that gene therapies bring: higher upfront prices, uncertainty about the long-term duration of clinical benefit and potential safety concerns.

Despite this, the promise of transformative value through long-term benefits and potentially curative treatments is motivating stakeholders to explore solutions to overcome these challenges and bring gene therapies to patients.

In a webinar held on 16 March 2021, prominent stakeholders, representing policy, patient and industry perspectives debated the key issues surrounding the adoption of gene therapies into health systems.

About this report

This summary report provides an overview of the different perspectives that were presented and the key themes that emerged from the webinar. Throughout we highlight quotes, encourage you to replay short clips from the event, and invite you to offer your perspective on the key topics.

Suggested reference:

Firth I and Hampson G, 2021. Gene therapies: are we ready? Event Summary. Office of Health Economics. Available from: https://ohe.turtl.co/story/gene-therapies-debate-report

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This event and report were commissioned and funded by Novartis Gene Therapies

The speakers

Mary Harney
Former Minister of Health for Ireland

Mary Harney is a former senior Irish politician.

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Annie Hubert
Managing Director, ESAH Pharma

Annie Hubert provides a wealth of experience

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Simone Boselli
Director of Public Affairs, EURODIS

Simone Boselli joined EURORDIS in April 2017.

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The different perspectives

The debate brought together three perspectives: policy makers, industry and patients. Each group has different priorities but they expressed optimism about gene therapies and confirmed that some of the challenges are shared between different groups.

PATIENTS Patients are excited for gene therapies but the delay in access feels long. There is concern that gene therapies are not affordable at current prices. The hope is that in the future there will be no delay between regulatory approval and patient access across Europe. Innovation in processes around regulatory assessment, value appraisal and reimbursement decision making need to keep up with scientific innovation.

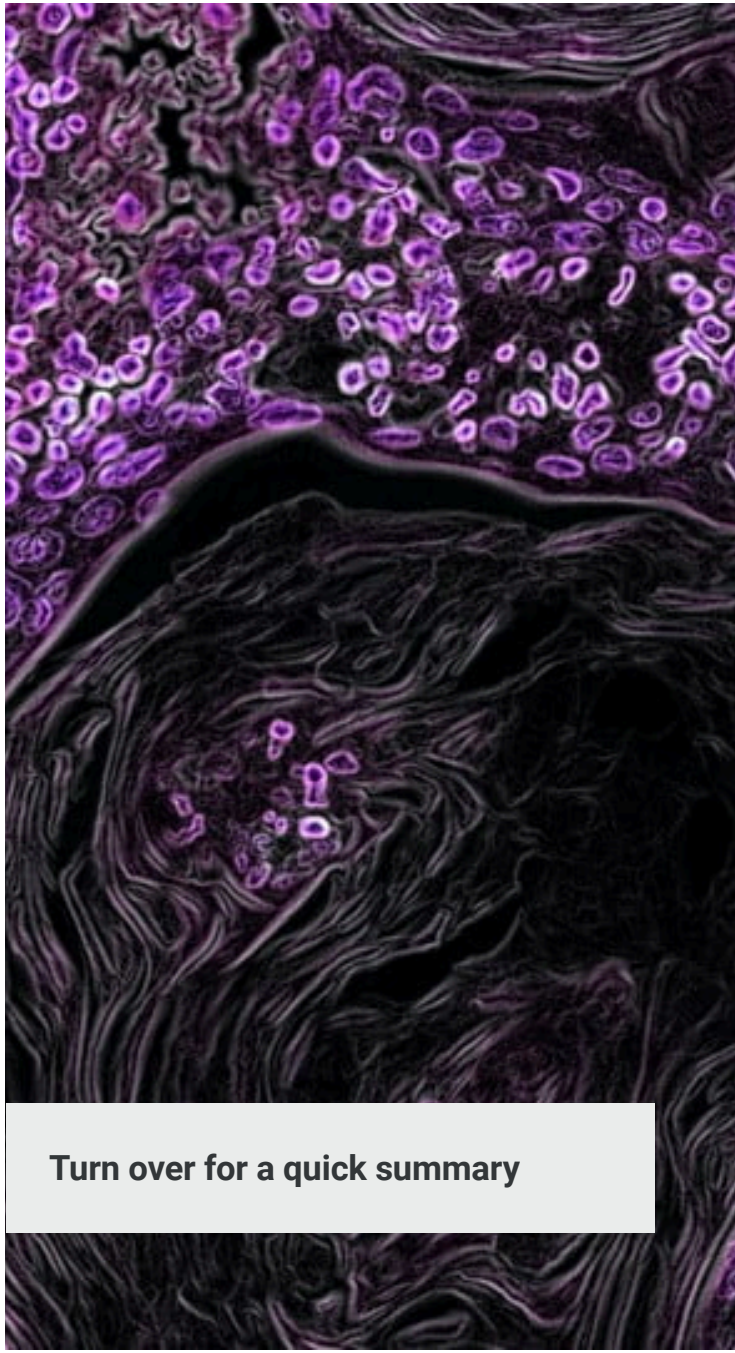
Watch the patient perspective **HERE**

POLICY There is a general lack of education among "the political class" about gene therapies with some people simply remarking "are those are the ones with the high price?". Short-term opportunity cost is a particular constraint for this group. Without data, we don't know what we are getting.

Watch the policy perspective **HERE**

INDUSTRY There is optimism now that some therapies are being approved and reimbursed. However there are practical barriers to widespread use of novel payment mechanisms including accounting rules and data infrastructure. A societal perspective for value assessment would help to capture the full value that gene therapies bring. Early dialogue is needed between all stakeholders to make sure that development decisions match the expectations of regulators, payers, health technology assessment (HTA) agencies, patients and clinicians.

Watch the industry perspective **HERE**



Turn over for a quick summary

Need a quick summary?

TAKEAWAY 1 Are we ready? It depends who you ask
Patients, regulators and clinicians are ready for gene therapies. HTA agencies are gaining experience. Policy makers and payers are not ready. Some people may be anti-gene therapy because of a fear of this new technology.

TAKEAWAY 2 Evidence generation is key
Evidence generation in real world settings will be needed to overcome uncertainty around clinical outcomes. Registries, electronic health records all need to be adapted for this.

TAKEAWAY 3 There is a role for cross-border alignment, but what is it?
Speakers looked to collaboration across Europe to overcome some of the barriers to gene therapies. Allowing patients to travel for treatment, EU data standard setting, as well as collaboration to remove European legislative barriers to gene therapies.

TAKEAWAY 4 There are practical and legislative barriers to progress
Science has delivered transformative therapies, but there are practical barriers to delivering them to patients. Some of the barriers discussed by speakers were:

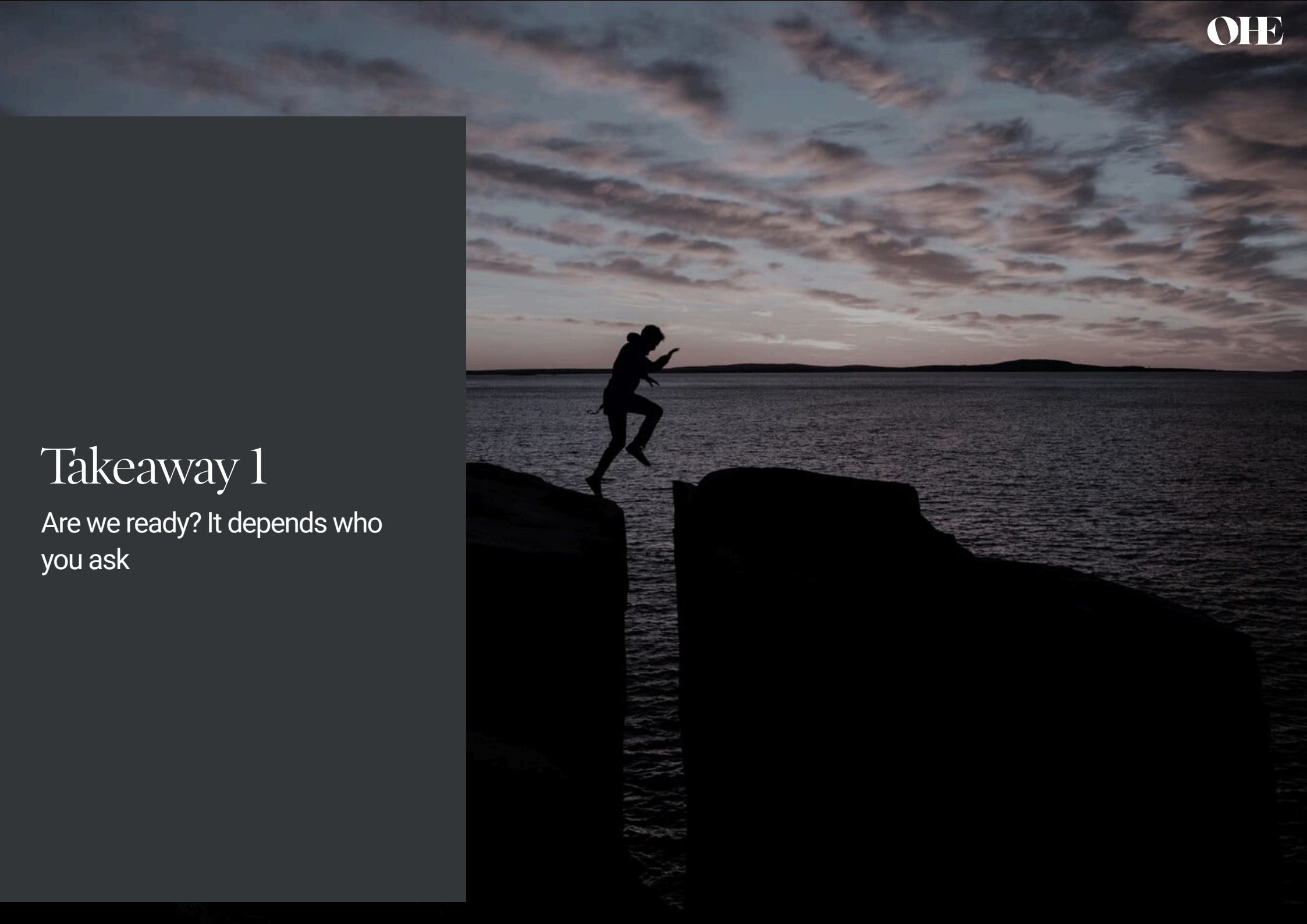
• Lack of strong data systems for monitoring outcomes
• Accounting standards that make annuity payments difficult
• HTA process that are not ready to assess the full value of gene therapies.

TAKEAWAY 5 Collaboration between *all* stakeholders is needed
Early collaboration between all stakeholders is needed to make sure plans are in place to limit delays in access. Companies, regulators, HTA agencies, payers as well as patients and clinicians all need a seat at the table.

Watch the chair, Adrian Towse, summarise the key discussion points from the debate

Takeaway 1

Are we ready? It depends who you ask



Are we ready? It depends who you ask

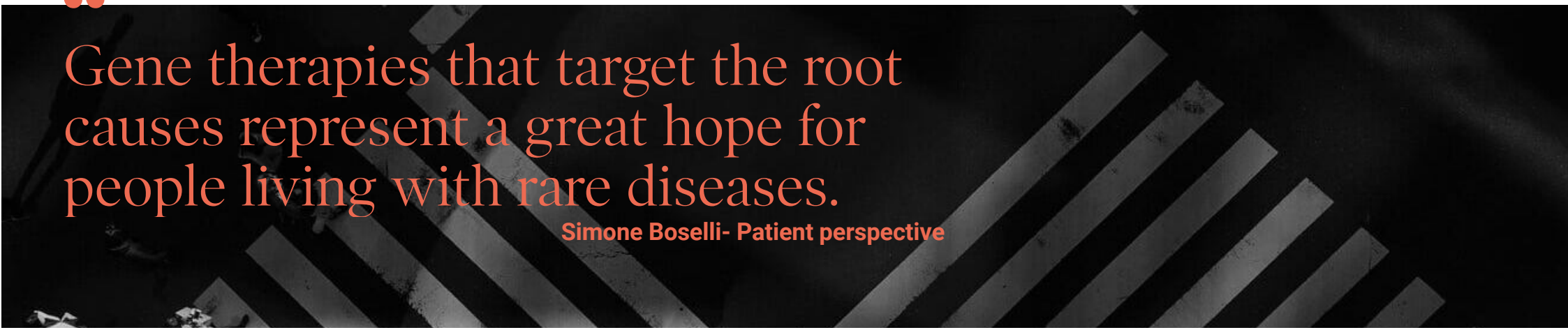
Quick summary
Patients, regulators and clinicians are ready for gene therapies. HTA agencies are gaining experience. Policy makers and payers are not ready. Some people may be anti-gene therapy because of a fear of this new technology.

Patients and clinicians are ready

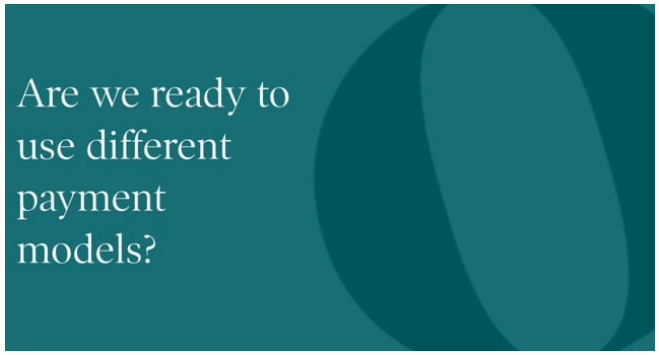
Patients are ready for gene therapies. They have high expectations and they are empowered to seek transformative therapies. Patients are interested in reducing delays to accessing gene therapies

Regulators and HTA bodies are gaining expertise

A number of gene therapies have been approved and reimbursed in recent years. Through this process regulators and HTA bodies have gained expertise. This expertise will be useful in the future as new gene therapies come to market.



> “Gene therapies that target the root causes represent a great hope for people living with rare diseases.
>
> **Simone Boselli- Patient perspective**



The "political class" are not ready

Politicians and policymakers are risk averse and have many other concerns. This group also focus on short-term budget cycles and they are not used to considering opportunity costs or the potential cost savings a technology may bring over the long-term.

There is also a lack of understanding of gene therapies among policymakers and politicians.

> We don't have unlimited resources so it is always about making choices, and if you do A you can't do B.

Mary Harney- Policy Perspective

Some people may be anti-gene therapy

People working closely with gene therapies, who understand the technology and its limitations could easily forget that for complicated and invasive technologies, public opinion could sour.

Similar to anti-vax and anti-GM crop movements, there is a potential for a backlash against gene therapy if cautiousness around safety morphs into disinformation and speculation.

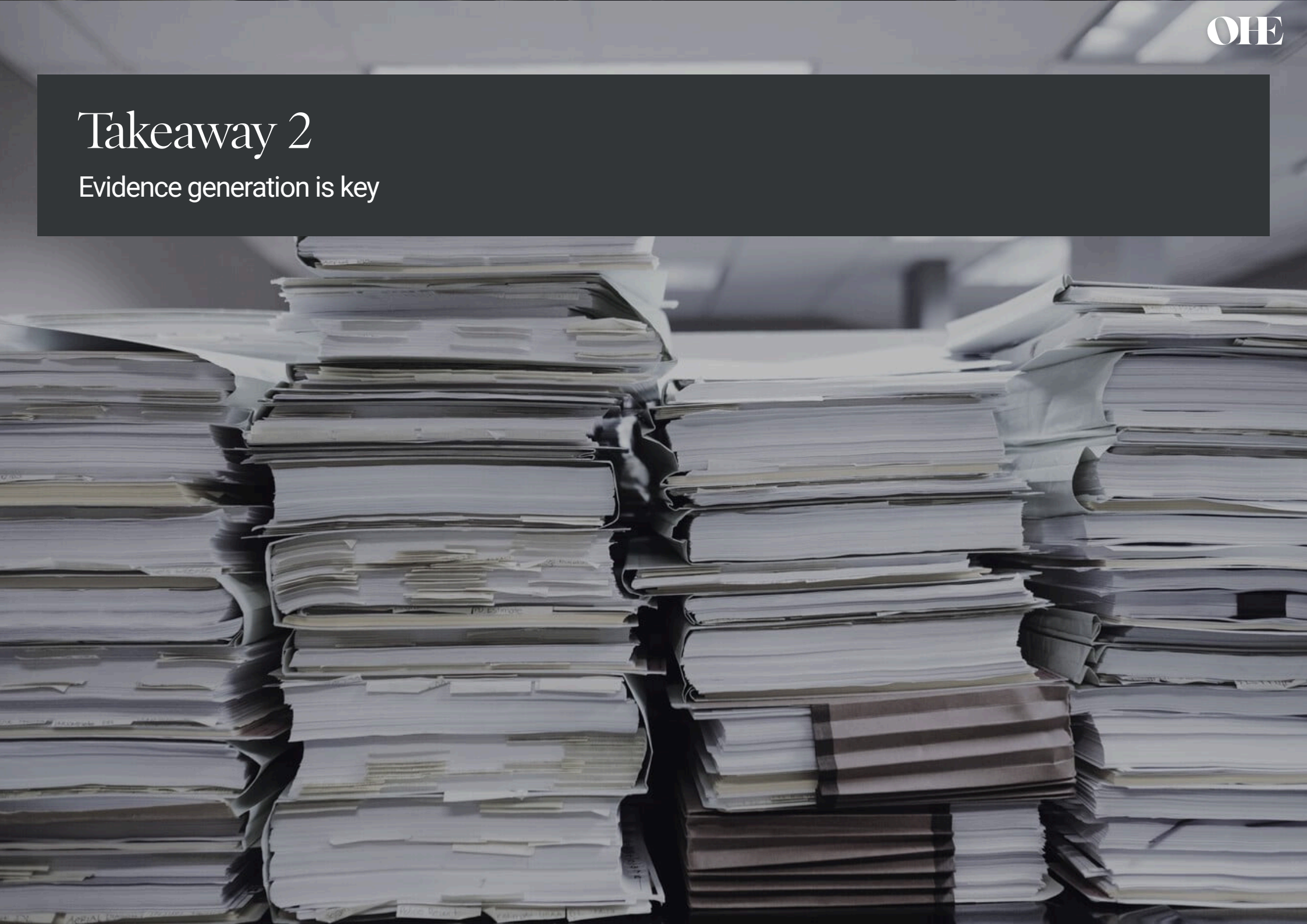
Have your say. Are we ready for gene therapies?

- Yes
- Not yet

SEE RESULTS

Takeaway 2

Evidence generation is key



Evidence generation is key

Quick summary
Evidence generation in real world settings will be needed to overcome uncertainty around clinical outcomes. Registries and electronic health records all need to be adapted for this.

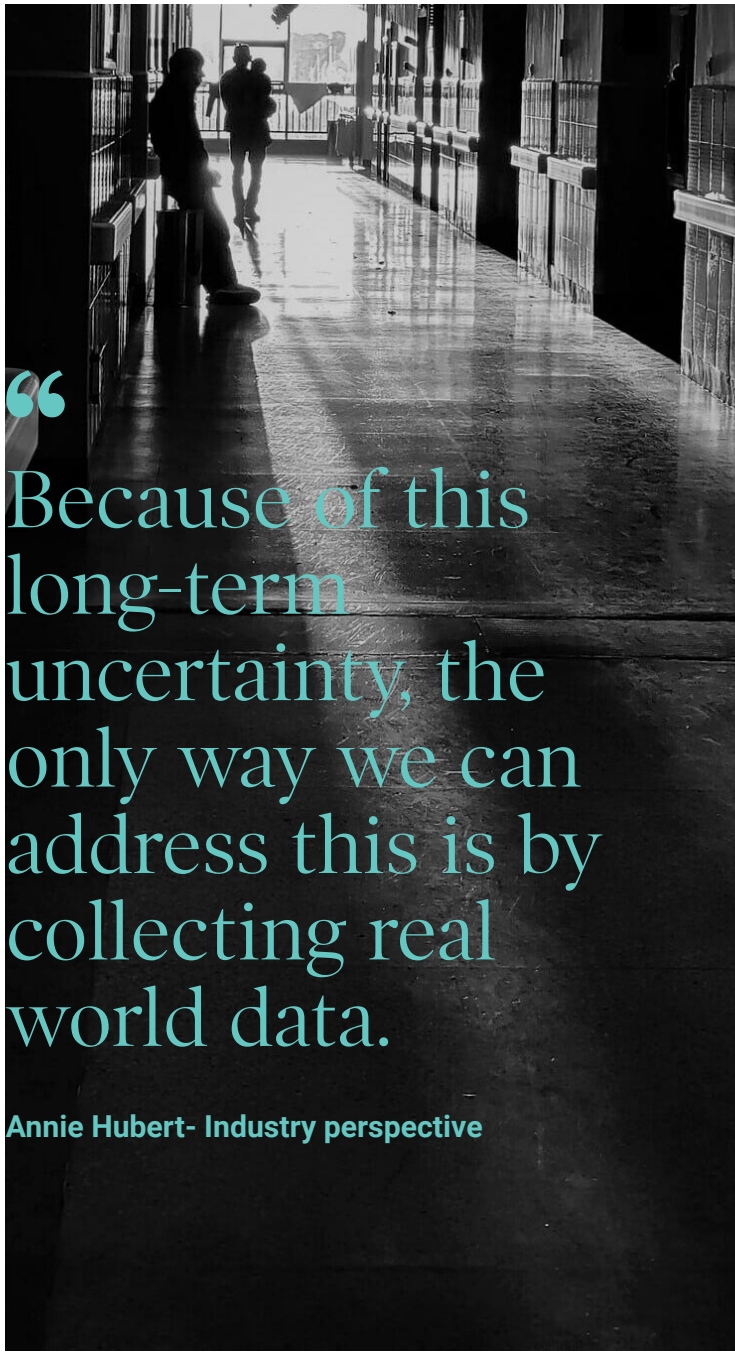
> “Everything hangs now with the data; a lack of data will be used to block or delay payment for these therapies if we don’t agree on the information we’re looking for.

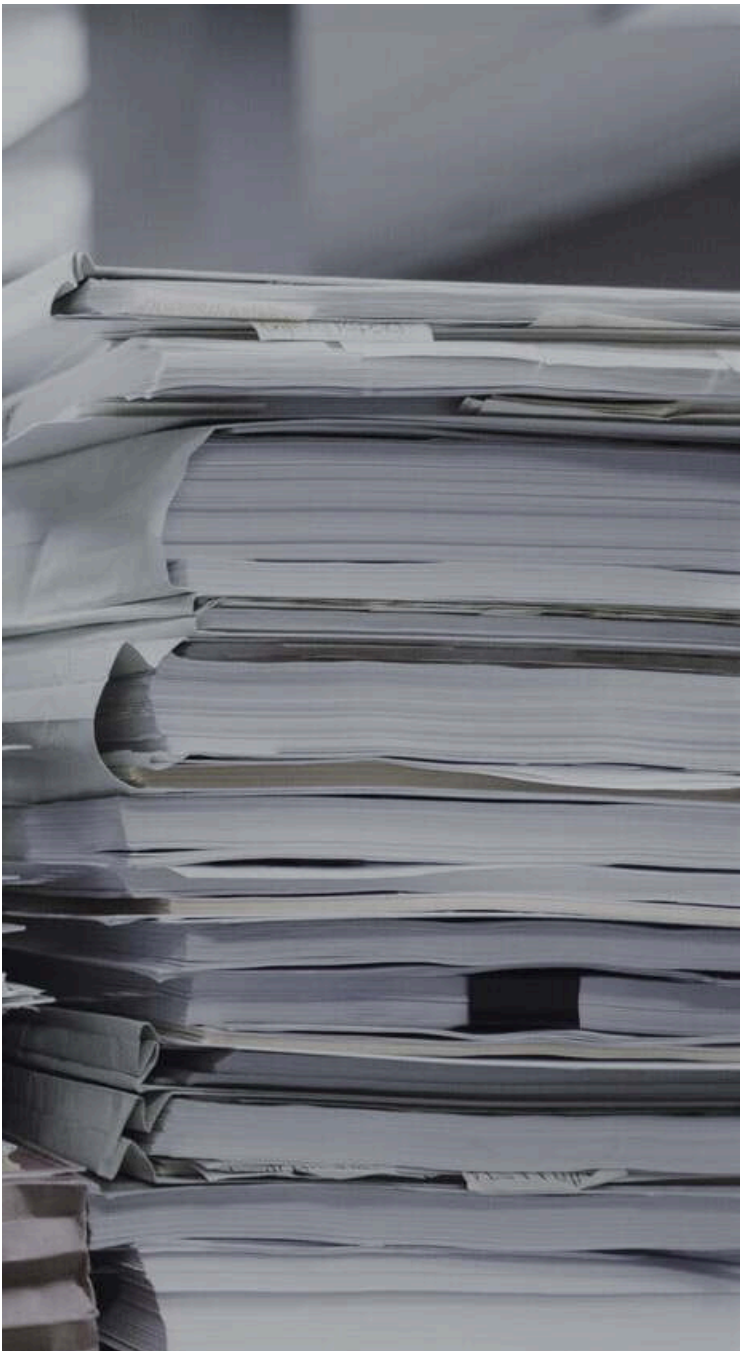
All three speakers focussed on the clinical uncertainty in safety and efficacy associated with gene therapies. This poses a problem particularly for payers and HTA agencies for whom value assessment is difficult with high uncertainty.

Electronic health records have not been set up to capture data to inform continued assessment of efficacy, safety and value after approval. Some countries and health systems are better equipped than others to handle this evidence generation requirement.

EU level guidelines on data standards would help health systems to gather data that is standardised and can be used to make decisions about efficacy and safety at a national level.

Real world data can be leveraged for the implementation of innovative payment mechanisms through outcomes-based





payments. Outcome-based payments allow payers to pay for outcomes, thereby reducing the risk of making the wrong reimbursement decision and reducing the upfront cost for payers.

Different stakeholders need different evidence

Regulators are adapting to manage different evidence packages for gene therapies and are motivated to approve gene therapies quickly because of the potentially transformative health gain. However this doesn't solve the problem of uncertainty as HTA agencies and payers need different evidence, on long-term value, to make decisions.

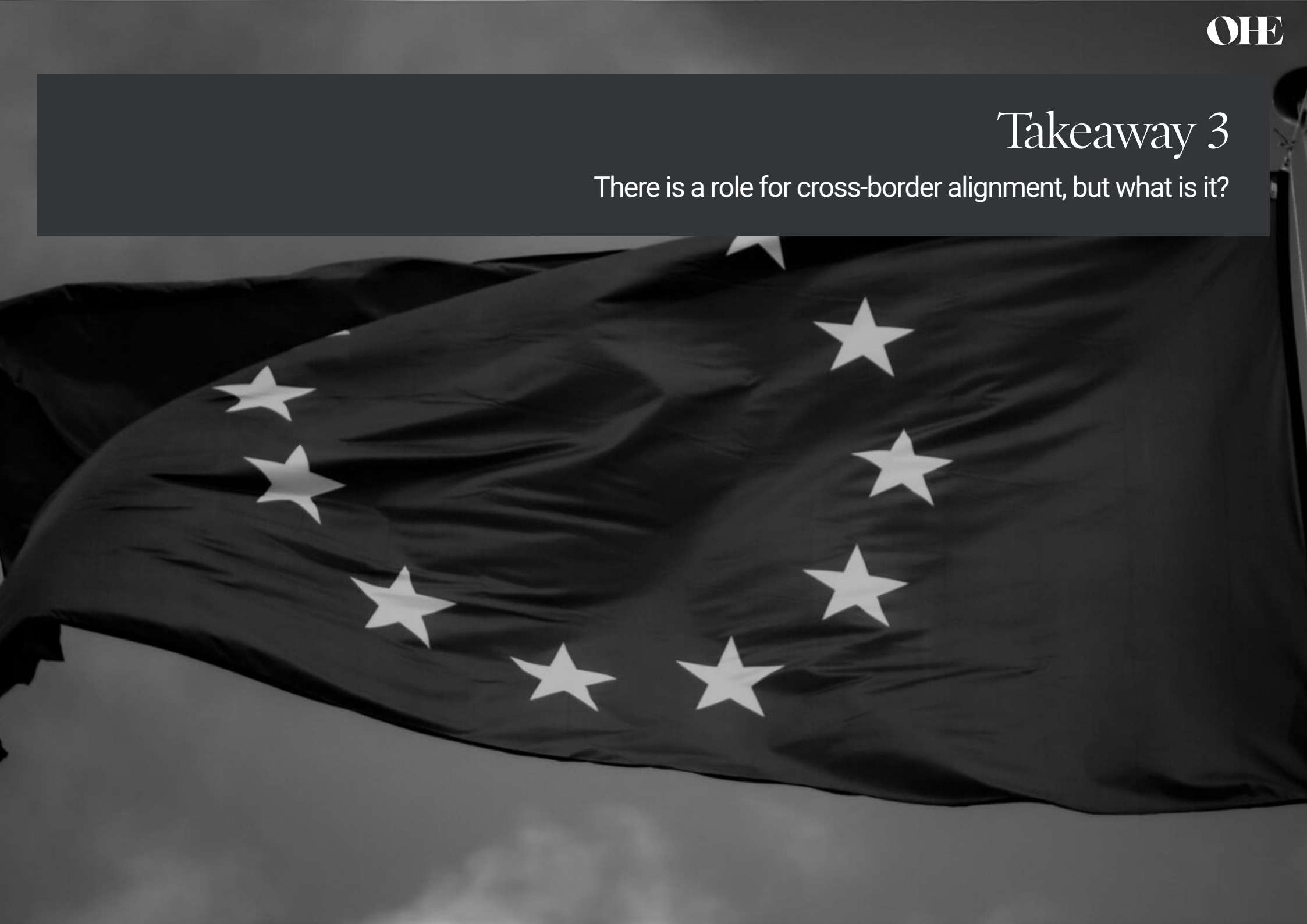
In addition, the way that evidence is collected is important for payers looking to use that data to form the basis of outcomes-based payment models.

A recognition that evidence generation requirements may be different across stakeholders is important for designing systems and processes to gather real world evidence. More on this in takeaway 5.

Data is key

Takeaway 3

There is a role for cross-border alignment, but what is it?



There is a role for cross-border alignment, but what is it?

Quick summary
Speakers looked to collaboration across Europe to overcome some of the barriers to gene therapies. Allowing patients to travel for treatment, EU data standard setting, as well as collaboration to remove European-level barriers to gene therapies.

Pan-European centres of excellence may be the future for gene therapy

For specialised technologies like gene therapies, it may not be plausible or necessary for every country to deliver them within their health system. Patients may be able to travel across borders for care in a similar way that they do within countries for other forms of specialist care.

There are many practical challenges to cross-border care. For example, the European Directive for cross-border care is not well suited for gene therapies because it requires patients to pay the upfront cost which is not practical for many patients.

The EU has role to play in standard setting for gene therapies

Countries are looking for European standards in terms of regulatory standards , data collection and (clinical) value assessment.

Some promising initiatives have already been developed. The European Health Data Space was mentioned as a promising step in the right direction that could be leveraged or piloted for gene therapies given the immediate need and clear application in the context of gene therapies.

> “There is a lack of a European Platform for collecting real world evidence where there are common standards for collecting the data, analysing and interpreting the data and standardising the knowledge.

Annie Hubert- Industry perspective

Joint procurement may not the answer

Individual member states want to keep their autonomy in relation to health spending. Where European collaboration is valuable is likely to be in mechanisms to reduce uncertainty and to reduce the risk for payers.

> “Member states guard their self-determination and their competence when it comes to health. Where I think Europe has a role to play is on the regulatory side trying to provide some consistency.

Mary Harney- Policy perspective

European level barriers need to be removed

The speakers discussed a number of examples of barriers that exist at the European level. For example the Directive on cross-border healthcare, which is not working well for gene therapies, or the European system of accounts (ESA) that make annuity payments (split payments) difficult or impossible.

For that reason, dialogue at the European level will be important to ensure that legislation doesn't have an unintended impact on limiting the adoption of gene therapies in Europe.

Where can pan-European collaboration add most value?

- Regulatory standards
- Data standards and governance
- Value assessment
- Joint procurement

SEE RESULTS

> “Europe has a role to play in investing in this infrastructure to allow the scientific advancement to be available to patients

Simone Boselli- Patient perspective

Takeaway 4

There are practical and legislative barriers to progress

There are practical and legislative barriers to progress

Quick summary

Science has delivered transformative therapies, but there are practical barriers to delivering them to patients. Some of the barriers discussed by speakers were:

- Lack of strong data systems for monitoring outcomes
- Accounting standards that make annuity payments difficult
- HTA processes that are not fit to assess the full value of gene therapies

Data systems aren't ready

There was significant discussion during the debate about the limitations in data systems to collect data to address the uncertainty surrounding the long-term efficacy and safety of gene therapies.

Electronic health records are not designed to capture outcomes that can be used to make reimbursement decisions. Registries, that are set up to capture data on individual diseases or disease areas, are not flexible for new disease areas.

Speakers agreed that real world evidence generation was a necessity for the adoption of gene therapies and that there could be a role for cross-border alignment on standards and data platforms to overcome this barrier.

Accounting standards could limit annuity payments

European system of accounts, for both companies and for governments, may restrict the use of annuity payments which could be used to spread the upfront cost of gene therapies.

> “There are other barriers, such as accounting systems in Europe which really are a barrier for the adoption of spread payments over time.
>
> **Annie Hubert- Industry perspective**

Health Technology Assessment may need to be adapted for gene therapies

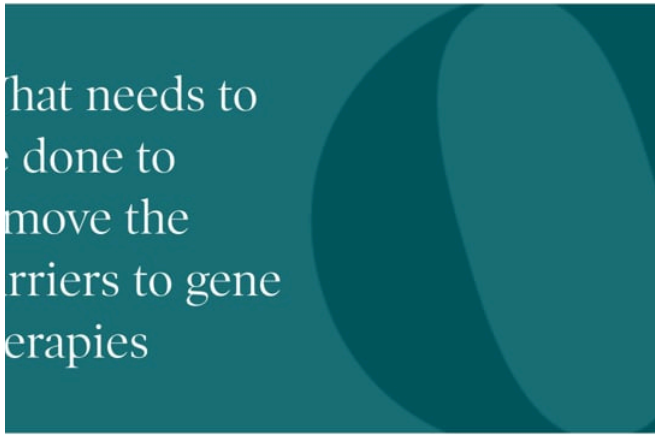
Health Technology Assessment (HTA) processes and other processes such as budget impact evaluation may not be fit for purpose for gene therapies.

What is an annuity payment?

The overall cost is paid in instalments, spreading the cost of a product or service over multiple budget cycles. Annuity payments can be conditional on a patient meeting certain predefined clinical outcomes. These are called outcomes-based payments.

Speakers agreed that HTA should take account of societal benefits of gene therapies. This would require HTA processes to be adapted to incorporate other forms of value in addition to the health gains and costs that are traditionally considered.

For example, for childhood diseases which require parents to become full-time carers, a treatment that removes that need has a much greater value than just the health gain to the child, there are also benefits for the parents and to wider society as a result.



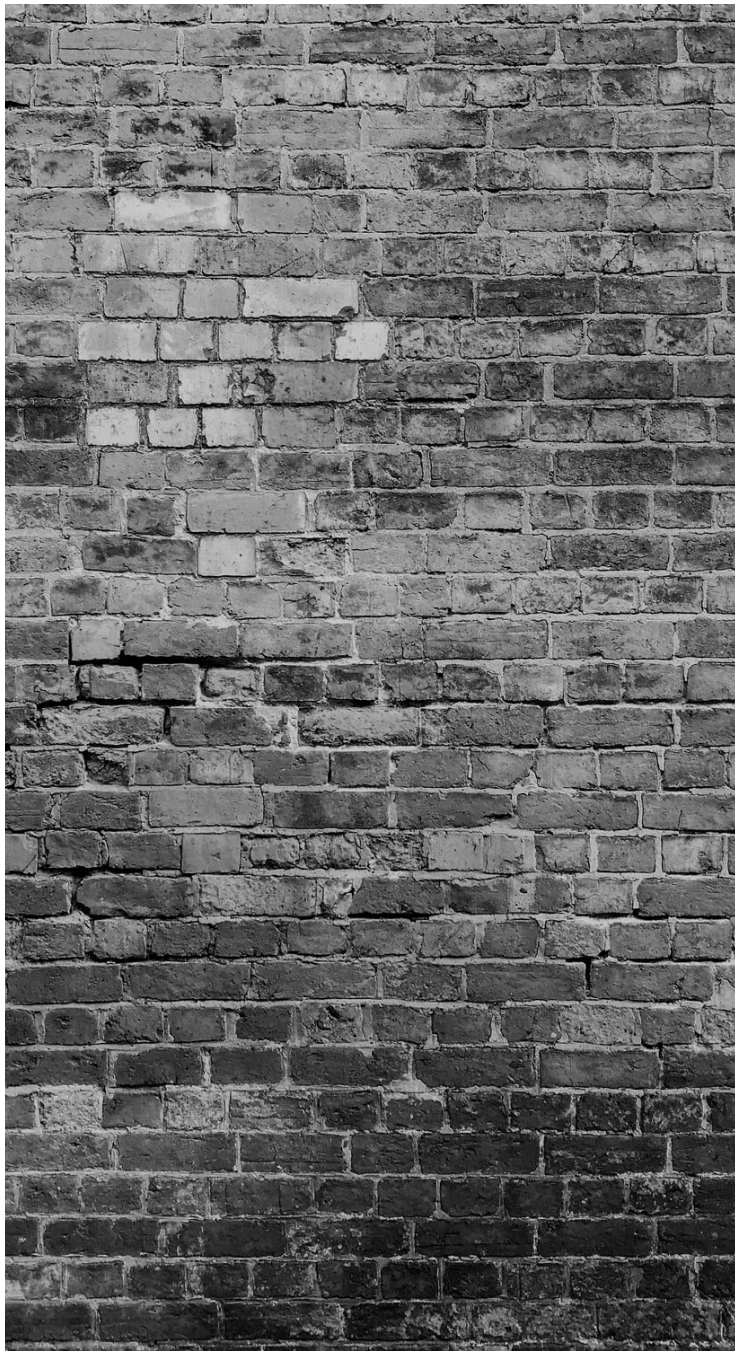
> I think at the moment we are in an incredible window of opportunity to translate the scientific advances, but we need to accompany them with innovation in processes not only in the way we pay but also the way we assess the value.

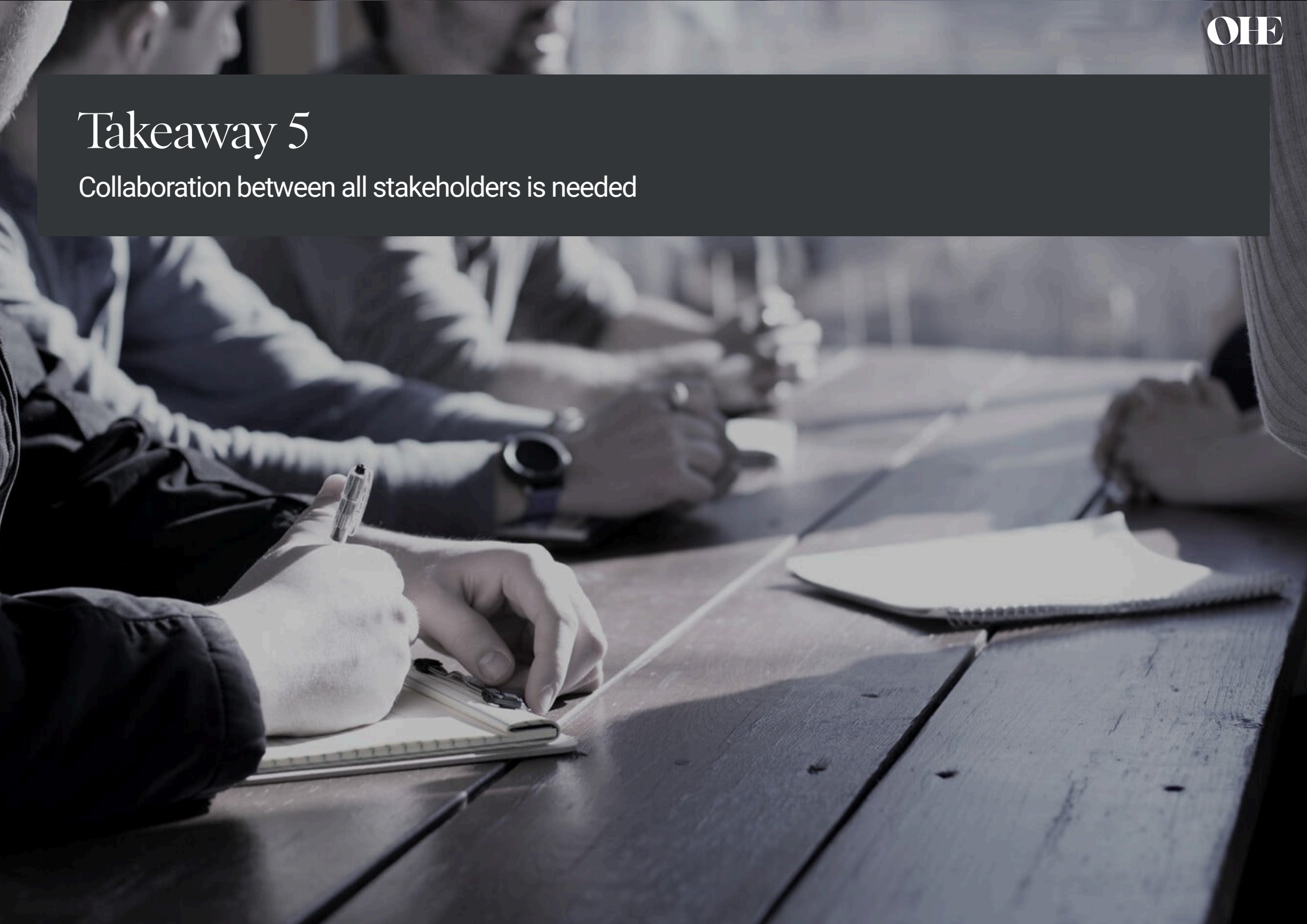
Simone Boselli- Patient perspective

How can HTA be adapted?

If you are interested in learning more about adapting HTA to incorporate other elements of value, take a look at these OHE publications.

- Assessment and appraisal of regenerative medicines
- HTA for ATMPs
- International comparison
- Cost-effectiveness thresholds

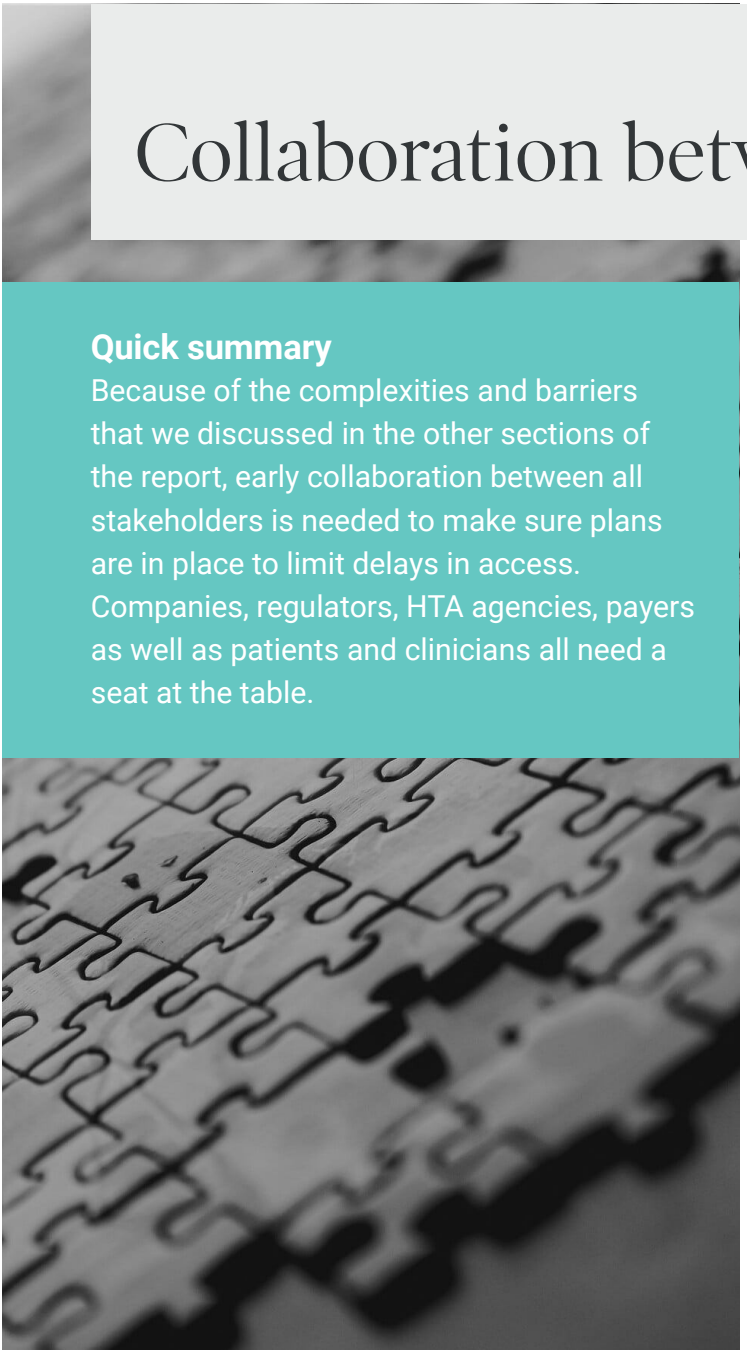




Takeaway 5

Collaboration between all stakeholders is needed

Collaboration between *all* stakeholders is needed



Quick summary

Because of the complexities and barriers that we discussed in the other sections of the report, early collaboration between all stakeholders is needed to make sure plans are in place to limit delays in access. Companies, regulators, HTA agencies, payers as well as patients and clinicians all need a seat at the table.

Making use of early dialogue

Regulators, payers, HTA agencies and payers are all interested in different aspects of a technology. As a result, early dialogue is important to ensure that the data to be collected will meet expectations and needs from all stakeholders.

There is a lack of information in the policy space

There is a lack of evidence and information for policymakers around gene therapies and how processes can be adapted to accommodate them.

> If we are to understand and make room for gene therapies, then we have to understand each others' perspectives.

Mary Harney

Informing and engaging policymakers on gene therapies will be important. It will be particularly important where barriers to adoption require legislative or policy changes.

> The key step-change that is needed is the use of early and iterative dialogue between regulators, HTA assessors, payers, with the due inclusion of patients and clinicians

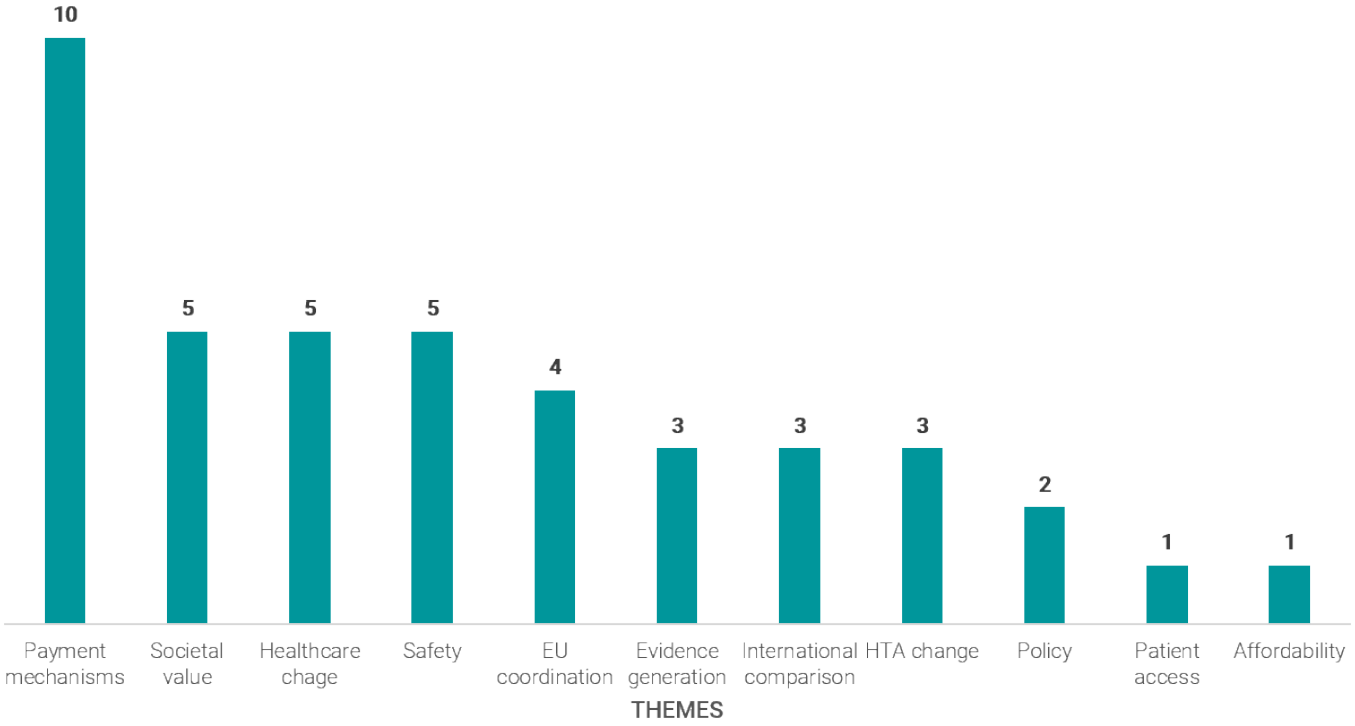
Simone Boselli

What did the audience think?

The questions from the audience can tell us a lot

What did the audience think?

An audience of over 300 delegates posed 38 questions and registered over 200 votes to raise the profile of the questions they found most interesting. Questions focussed on payment mechanisms, societal value of gene therapies and healthcare changes that would be needed to accommodate gene therapies. Some key words and phrases from the questions are mapped to the right of this page.



There were lots of topics we didn't have chance to cover

Lots of interesting health economic topics were raised that we ran out of time to cover. There were many questions about how different payment models can be used for gene therapies.

If you're interested in these topics, here are some great OHE reports for more in depth analysis.

Making outcomes-based payment a reality in the NHS

READ REPORT

Early Experience with Health Technology Assessment of Gene Therapies in the United States: Pricing and Paying for Cures

READ BRIEFING

Gene therapy: evidence, value and affordability in the US health care system

READ PAPER

Paying for Cures: Perspectives on Solutions to the "Affordability Issue"

READ PAPER

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Which topic is most crucial for you?

- Payment mechanisms
- HTA change
- Safety
- EU alignment
- Evidence generation
- Patient access
- Affordability

SEE RESULTS

Thank you for reading

Gene therapies debate event summary

To enquire about additional information and analyses, please contact Graham Cookson CEO
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