Monitor Deloitte.

Accelerated Access Review: UK Mapping

For web publication

30th March 2015

This is independent research commissioned by the Accelerated Access Review. The views expressed are those of the authors and not necessarily those of the Accelerated Access Review.

casmi

The King's Fund

Dear Reader,

This report presents the work undertaken in Workstream 1 of the *Accelerated Access – Innovative Medicines and Medical Technologies Review* during March 2015. For this workstream the consortium of Monitor Deloitte, CASMI and King's Fund focused on describing the end-to-end 'pathways' for pharma, medtech and *in vitro* diagnostics by which innovative medical technologies pass from conception to widespread patient access, identifying key barriers to rapid NHS adoption of such innovations, challenges to Industry and opportunities to improve patient access. The overarching aim of this work was to improve patients' health outcomes and experience through faster access to efficacious medical innovations (including digital), not to expand the market for manufacturers of innovations *per se* or to promote innovation for its own sake.

We used extensive interviews, desk research and stakeholder workshops to build up our analysis of the pathways as they stand today and identify the perceived barriers to rapid adoption of innovation in the UK. As such this document is a single 'snap-shot' of how health economy participants view the current environment and may not reflect all the initiatives currently in place to address these barriers and challenges, either because the stakeholders we spoke with are not yet seeing results or because they are not aware of these programmes.

We interviewed a wide range of stakeholders from across the health economy, including Industry, the Public Sector, Academia and Charities. In the available time frame we were not able to focus on interviewing a significant number of frontline clinicians and this will be an important activity for the next phase of this work.

We note that many of the highlighted barriers are being addressed in whole or in part by a series of existing initiatives. The key initiatives that are not yet built into our analysis are:

- The NHS Five Year Forward View that committed to exploring new mechanisms for achieving quicker adoption of cost-effective innovation through partnerships, including with patients and voluntary sector organisations;
- The EU's Innovative Medicines Initiative to increase the speed of development of better and safer medicine through Europe's largest public-private partnership;
- The NHS Commissioning through Evaluation programme to examine real world clinical evidence in the absence of full trial data (e.g., expansion of the indication for use of stereotactic ablative radiotherapy beyond non-small cell lung cancer);

This document raises a series of interim questions regarding how best to improve NHS patient access to innovation, which will be addressed in much greater detail in the next phase of the *Accelerated Access – Innovative Medicines and Medical Technologies Review*. The next stage of the review will consult, review and recommend policies aimed at improving access.

Monitor Deloitte, CASMI and King's Fund

Contents

Executive Summary

Introduction

This report represents the work undertaken in workstream 1 of the Accelerated Access – Innovative Medicines and Medical Technologies Review. This workstream focused on the end-to-end pathways for pharma, medtech and *in vitro* diagnostics, identifying the barriers to rapid adoption of innovation, challenges to Industry and the opportunities to improve the pathways. For each type of technology reviewed the pathways were broken down into development, regulation, national reimbursement and local commissioning and adoption, so challenges and HMG influence levels on improvement opportunities could be assessed

Methodology

To develop the final report the outputs of four phases of work were combined:

Desk research	Interviews	Workshops	Synthesis
Insights gathered from: desk research, OLS, Deloitte, CASMI, King's Fund, case studies shared	Over 50 people interviews on from Industry, Public Sector, Academia and Charities	20 participants on 20th March 100 participants on 24th March 15 participants on 17th April	Led by the Deloitte team supported by CASMI and King's Fund

Cross-cutting challenges

Our research has highlighted four cross-cutting challenges for early patient access to innovative pharmaceuticals, medical devices and *in vitro* diagnostics:


Alignment across key stakeholders


Counterproductive incentives


Data, evidence & measurement


Culture

Additional challenges have been identified for all pathway phases by development, regulation, reimbursement and local adoption

Future proofing

In assessing how future proof are the pathways for digital health and precision medicine the following questions were asked: 1) What is the current pathway? 2) Is it fit for purpose? 3) What needs to change? Both pathways are currently relatively unclear: digital is not currently a pathway at all and the addition of a companion diagnostic complicates the existing pharma pathway. Neither are considered to be fit for the future. To future proof both pathways better data, evidence and measurement are required. In addition digital health needs to incorporate new funding models and precision medicine pathway needs to integrate the companion diagnostic into both the assessment and funding mechanisms. Future-proofing the pathways for other advanced therapies, such as cellular / genetic therapies, will require similar though substantial higher effort

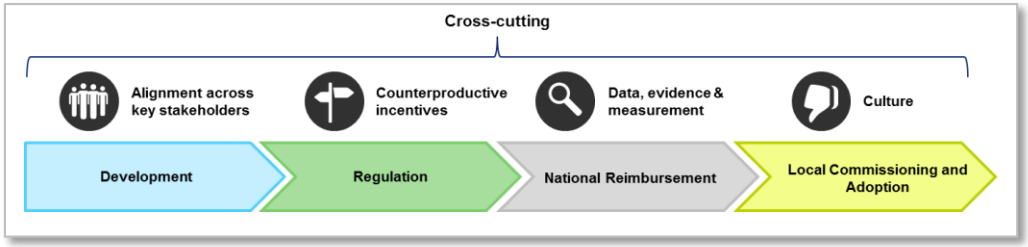
Opportunities to review in Phase 2

Twelve opportunities have been prioritised to take forward into Phase 2 of the review. These twelve areas include a forward-looking update of the end-to-end pathways for pharma, precision medicine, medtech and digital health. They also reflect the identified cross-cutting challenges: alignment across key stakeholders; counterproductive incentives; data, evidence and measurement; and culture. The remaining four opportunities relate to specific areas of importance at key parts of the pathway or the role of key stakeholders: patient engagement, the clinical development environment in the NHS, local adoption incentives, and the role of regional centres such as AHSNs

Challenges to early access

Throughout this report we consider two sets of access challenges for innovative pharmaceuticals, medical devices and *in vitro* diagnostics

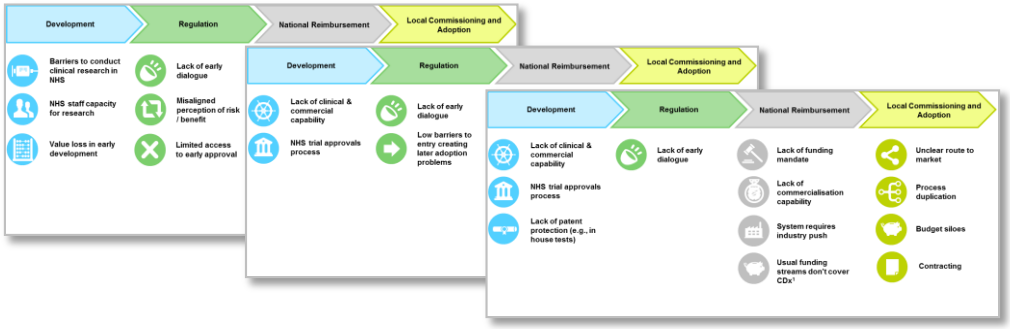
1 Cross-cutting challenges



Apply across whole end-to-end pathway

Apply across all 3 sectors

2 Sector & pathway-specific challenges



Vary by pathway stage

Vary by sector

5 Source: Stakeholder interviews, workshops and Deloitte analysis

Four key challenges

Our research highlighted four cross-cutting challenges for early access to innovative pharmaceuticals, medical devices and *in vitro* diagnostics

Alignment across key stakeholders

- Stakeholders and organisations **along the pathway are not aligned** with the **explicit goal** of working together to **bring innovations more reliably and rapidly to patients.** This reduces the adoption of innovative pharmaceuticals, medical devices and *in vitro* diagnostics. The problem is **most acute across and between national reimbursement and local adoption** and Industry understanding of NHS clinical priorities. It's also felt that **patients have limited opportunity** to have their **voice their views** along the pathway

Counterproductive incentives

- Counterproductive incentives, such as **internal budget siloes** within and between NHS organisations dis-incentivises the adoption of innovation where the **costs and value realised are not aligned**. Short financial **time horizons** (and a lack of easy vehicles to **invest in multi-year returns**) and an individual organisation's financial position, rather than a whole system view further **dis-incentivises investments that may save costs and improve outcomes at an aggregate level**

Data, evidence & measurement

- Evidence is **not being used as an effective enabler** to facilitate the development, reimbursement and adoption of innovative medicines and medical technology. There is a **lack of clarity (and often availability)** around the evidence that is required at each stage of the pathway, particularly in **medical technology and for SMEs**

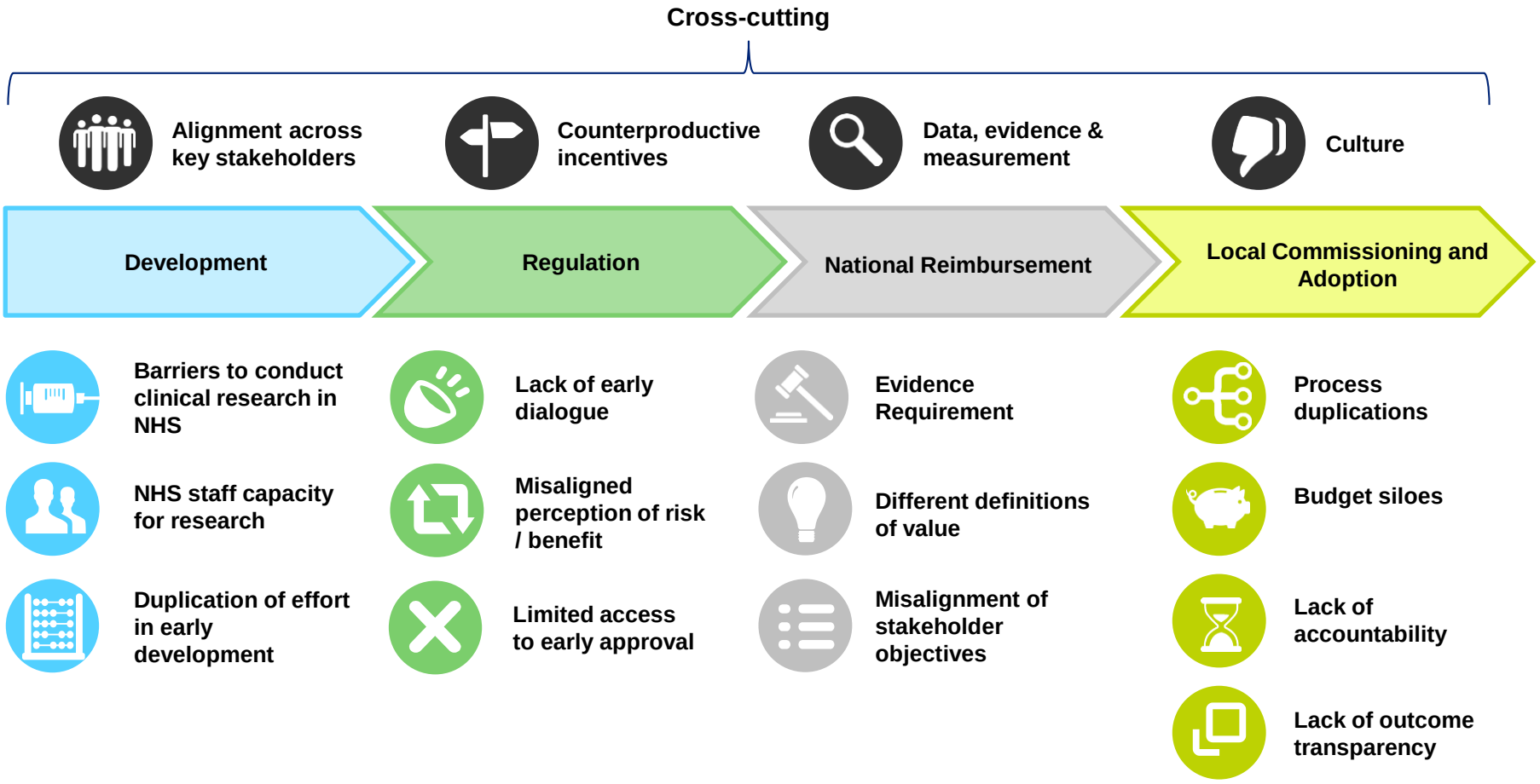
Culture

- **Lack of trust between NHS and Industry** is a key barrier to **more collaborative working** across the end-to-end pathway. Other cultural barriers to the adoption of innovative medicines and medtech include **local risk aversion, distrust of external data and evidence, non-constructive competition between NHS organisations and resistance / lack of capacity to implement significant change**

Source: Stakeholder interviews, workshops and Deloitte analysis

Challenges: Pharmaceuticals

Our research also highlighted a number of specific challenges to pharma by pathway stage



7 Source: Stakeholder interviews, workshops and Deloitte analysis

Challenges: Medical devices

Our research also highlighted a number of specific and different challenges to medical devices by pathway stage

Cross-cutting

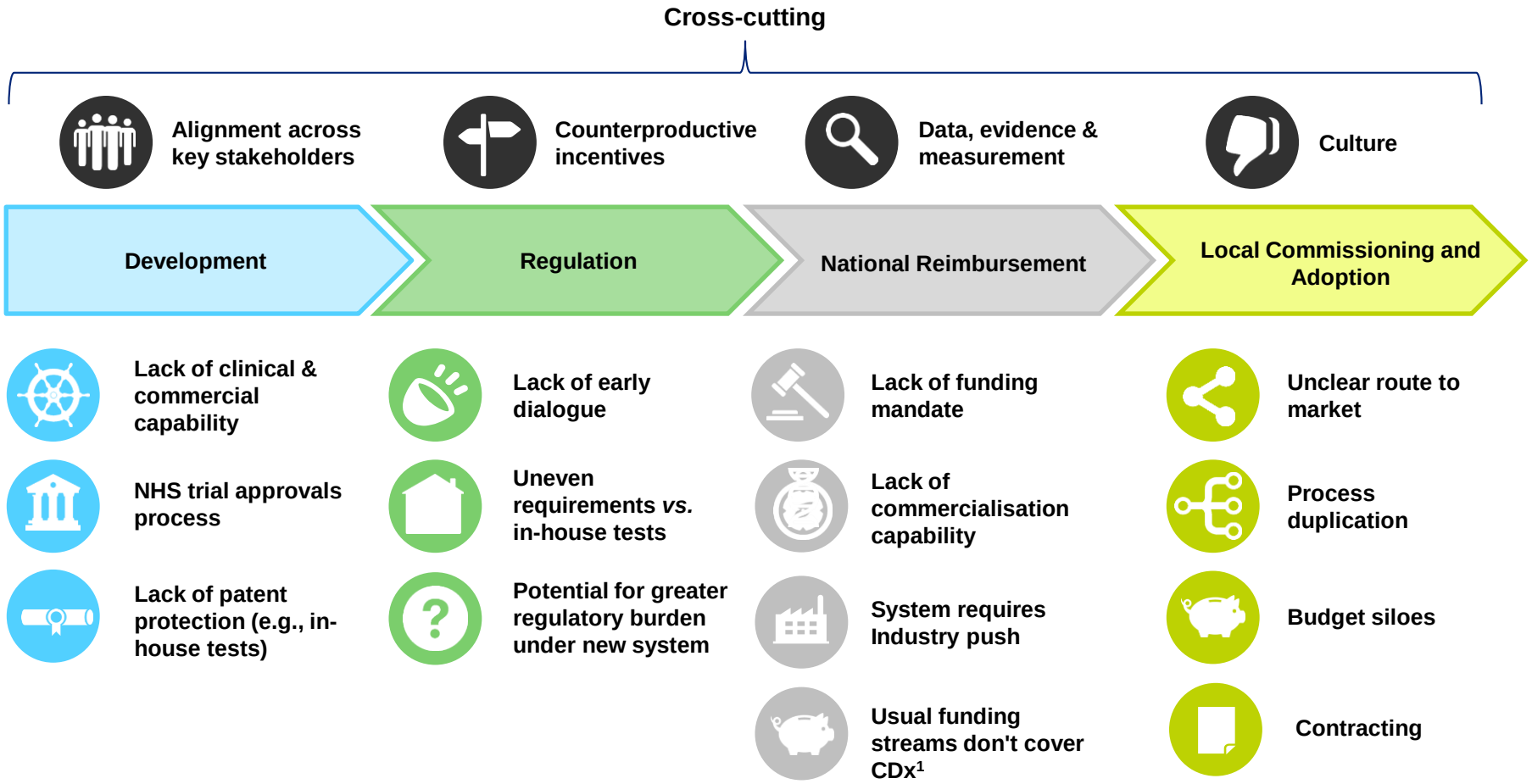
- **Alignment across key stakeholders**
- **Counterproductive incentives**
- **Data, evidence & measurement**
- **Culture**

Development	Regulation	National Reimbursement	Local Commissioning and Adoption
Lack of clinical & commercial capability	Lack of early dialogue	Lack of funding mandate	Unclear route to market
NHS trial approvals process	Low barriers to entry creating later adoption problems	Lack of commercialisation capability	Process duplication
		NICE turnaround times	Budget siloes
		System requires Industry push	Speed of pricing / contracting update
			SME expertise / scale

Source: Stakeholder interviews, workshops and Deloitte analysis

Challenges: *In vitro* diagnostics

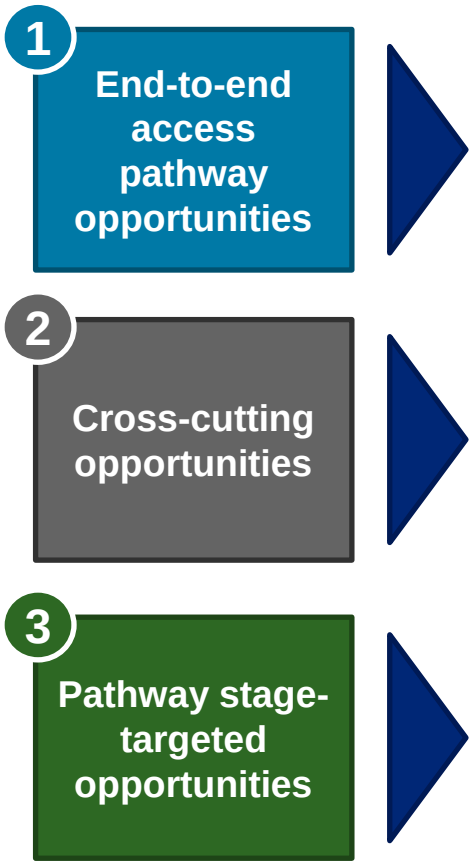
Our research also highlighted a number of challenges that are specific to *in vitro* diagnostics by pathway stage



Notes: (1) CDx = co-diagnostics; Source: Stakeholder interviews, workshops and Deloitte analysis

Opportunities for improved access

We have identified 12 priority opportunities for improved patient access to innovations, grouped into 3 areas



- Four opportunities that each address the patient access pathway for a specific set of innovative products
- Four opportunities that address each of the four identified cross-cutting challenges
- Four opportunities that address specific parts of the pathway for specific products, where research indicated a particular challenge

 Source: Stakeholder interviews, workshops and Deloitte analysis

Top areas for review in phase 2

Based on our research, interviews and workshops, we recommend the following opportunities to take forward into Phase 2

1. Further review of pharma pathway opportunities and future proof for agile pathways
2. Further review of medtech pathway to standardise and improve parity with pharma
3. Future proof for precision medicine pathway
4. Lightly regulate and develop systems to support adoption of digital health technologies
5. Review organisational mandates and alignment to support adoption
6. Funding of innovation, system incentives and contracting mechanisms in the NHS
7. Role of data to support the end-to-end pathway and adoption of innovation
8. Assess cultural barriers to collaborative working, e.g. facilitate earlier dialogue
9. Clinical development environment and incentives for NHS involvement
10. Reimbursement methodologies to incorporate early access and RWE
11. Promote patient engagement throughout the end-to-end pathway
12. Role of regional centres e.g. AHSNs as test beds and drivers of innovation

 Source: Stakeholder interviews, workshops and Deloitte analysis

Contents

Accelerated Access Review

The review will make recommendations to government on opportunities to accelerate access for NHS patients to innovate medicines and medtech

The Accelerated Access – Innovative Medicines and Medical Technologies Review (IMMTR) announced by George Freeman on 20th November 2014 will consider how the healthcare and regulatory systems can best respond and adapt to the new landscape of digital diagnostics, genomics and precision medicine. The review will consider how to speed up access for NHS patients to cost-effective new diagnostics, medicines and devices.

With initial findings being reported in late Summer, the work will identify key challenges across the end-to-end pathway and provide opportunities for change to accelerate patient access of upcoming innovative medicine and medtech in the current pathways. Specifically the review will achieve the following outcomes:

- *Identify key priorities for action*
- *Provide implementable suggestions for change to accelerate innovation access*
- *Develop a sustainable framework to support and drive medical innovation*
- *Consider the long term landscape for innovation adoption*

There are a number of workstreams contributing to the review and this report focuses on Workstream 1. The objective of workstream 1 is to assess the As-Is environment in the UK and the barriers to getting patients earlier access to medical innovation.

Workstream 1 objectives

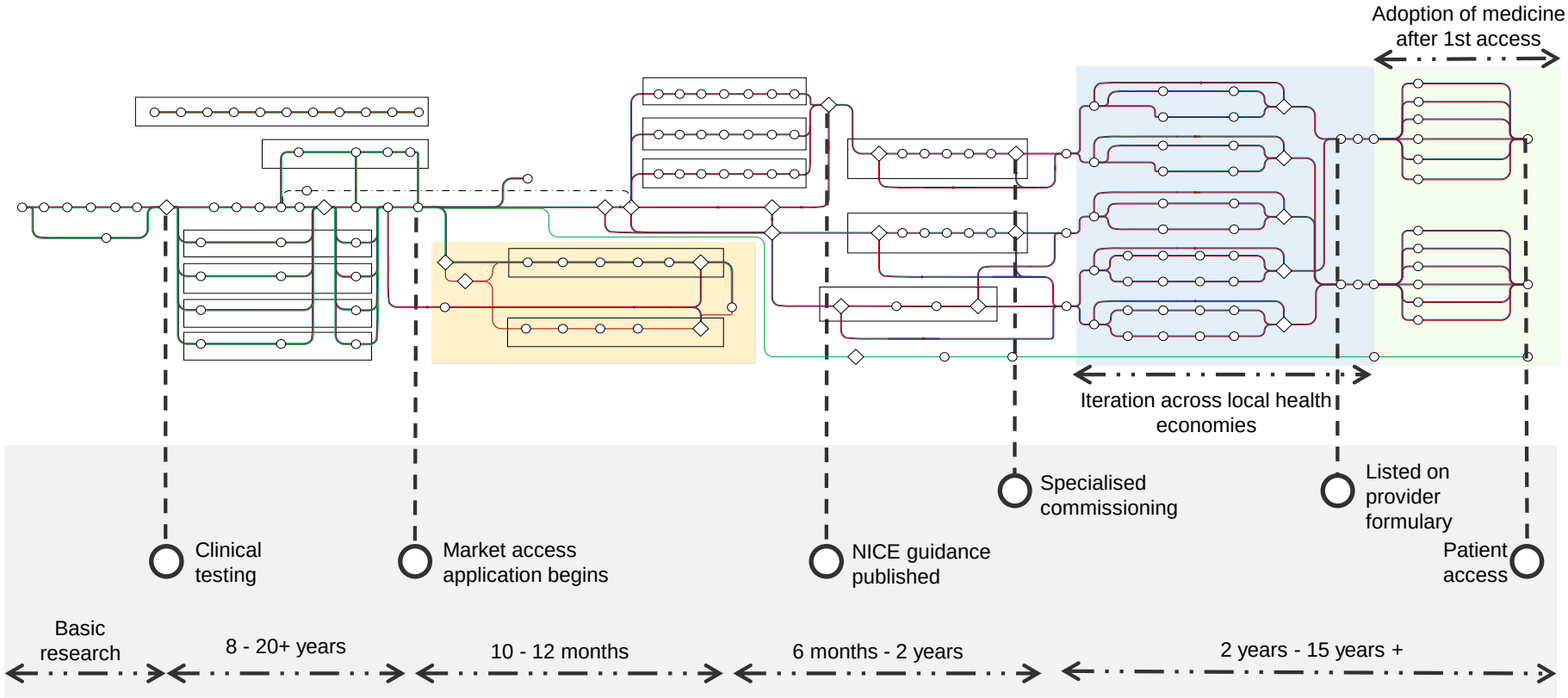
There were 5 key aims of workstream 1; these aims are covered in this report with the associated detailed end-to-end pathways created to support workstream 1

1. To map the current processes and pathways through which innovative medicines, devices and diagnostics are assessed from proof of concept through regulation, cost-effectiveness assessment and adoption in the UK
2. To understand how these processes and pathways work in practice
3. To identify issues, barriers and opportunities within the UK's current approach, and highlight how and when innovation creates the opportunity to evolve the processes and pathways now and in the future
4. To identify particular areas within current pathways and processes that will be challenged by the new types of product and environment, assess the degree to which the system is future-proofed and identify priority areas for change
5. To identify the key features which allow rapid development in 'analogous sectors (innovative, high market failure, high upfront costs, large-scale public use), and to explore whether any of these features – or lessons learnt from them – could be effectively mirrored in the development pathways for medicines, devices and diagnostics

Whilst regulatory issues will be considered UK-wide, cost effectiveness and adoption will be examined on an England-only basis

Pharmaceutical pathway summary

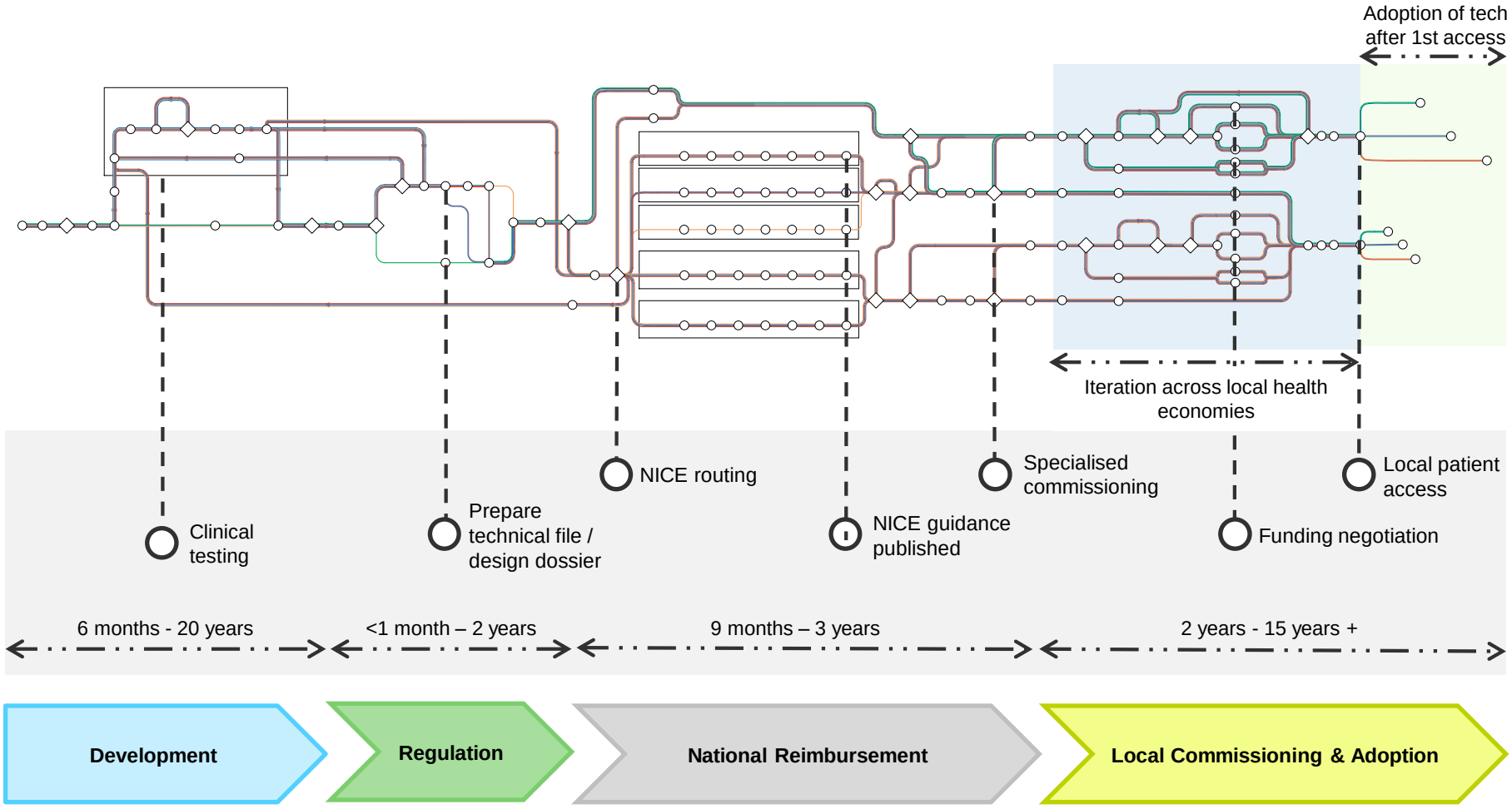
This is a linear representation of a process that is not perfectly linear. The pharmaceutical pathway has not significantly changed for many years



 Source: Stakeholder interviews, workshops and Deloitte analysis

Medical devices pathway summary

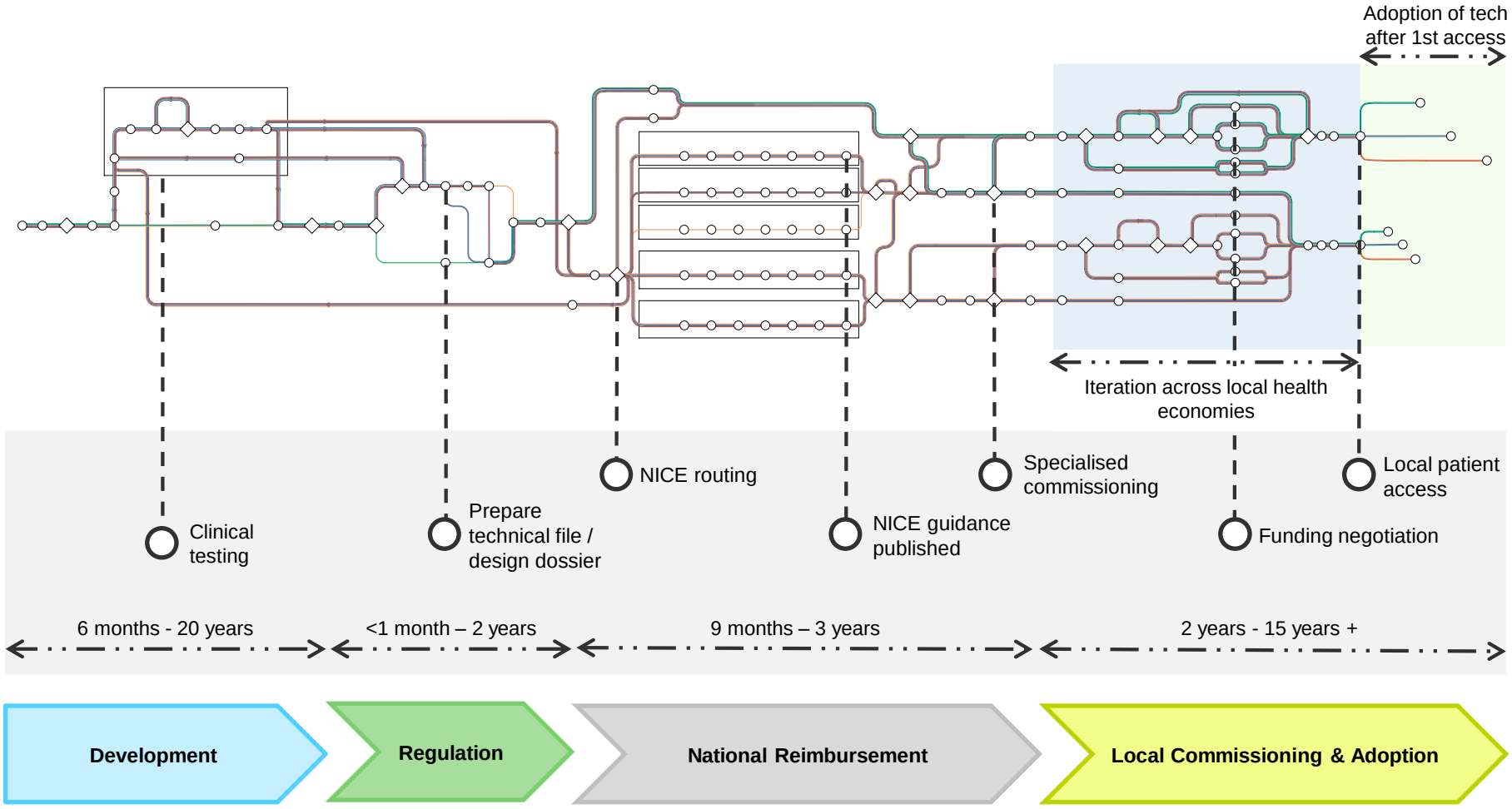
At a high level, the medical devices pathway has fewer component processes than the equivalent pathway for innovative pharmaceuticals



Source: Stakeholder interviews, workshops and Deloitte analysis

In vitro diagnostics pathway summary

Currently, the route to patients for *in vitro* diagnostics does not differ significantly from that of other medical devices



 Source: Stakeholder interviews, workshops and Deloitte analysis

How to read this document (1/2)

We have structured this document around product types and pathway stages

This review considers the current patient access pathways for three key product types

A

Product type

Pharmaceuticals

Medical Devices

In vitro diagnostics[1]

Each pathway has been broken down into four stages

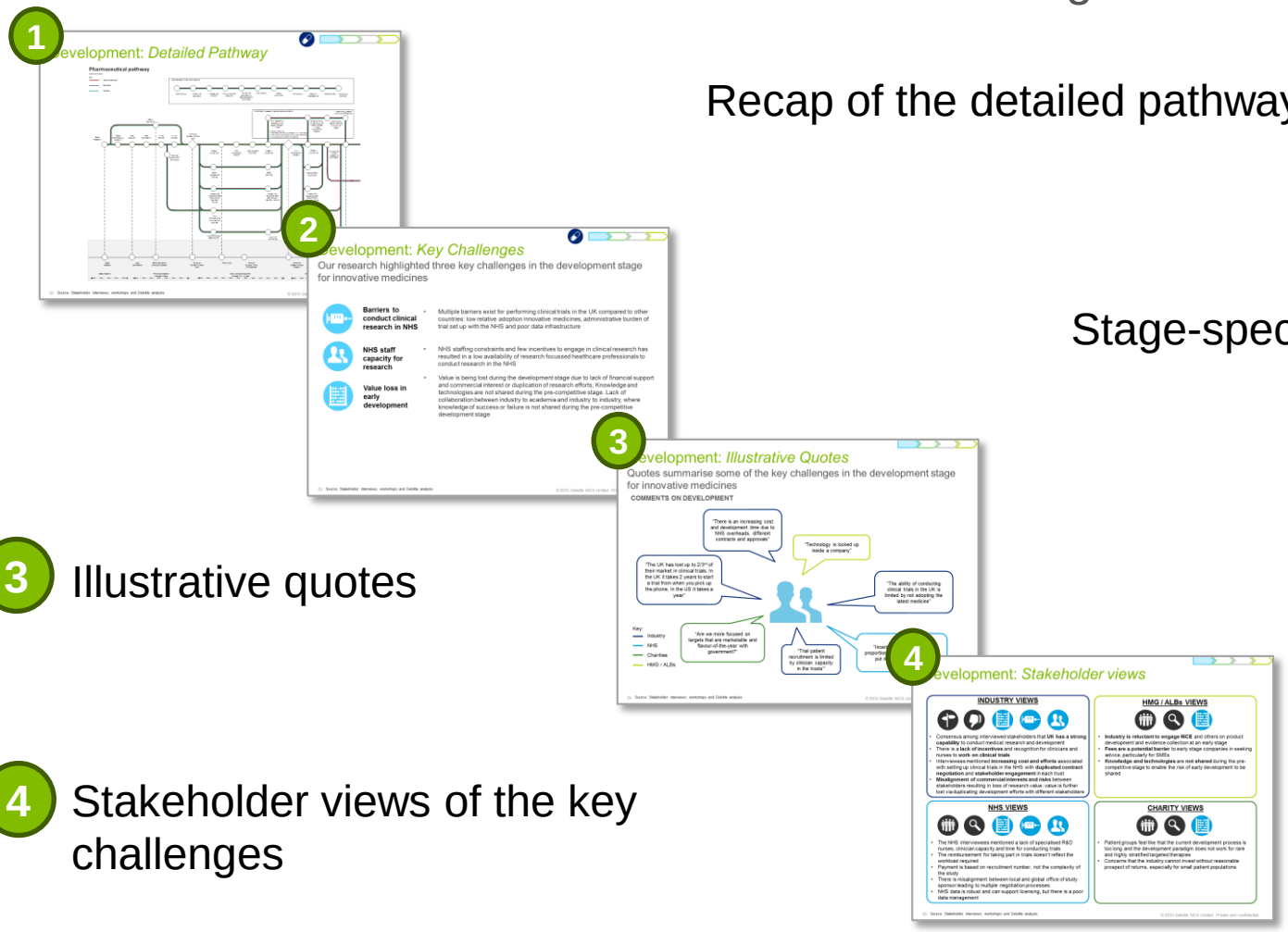
B

Pathway Stage

Notes: (1) *In vitro* diagnostics have been considered as a distinct group as they fall under a distinct Directive to medical devices. Other kinds of diagnostics (e.g., CT scanners) are classed as medtech by the regulatory framework

How to read this document (2/2)

Each detailed section below contains the following slides:



1 Recap of the detailed pathway for that stage

2 Stage-specific challenges

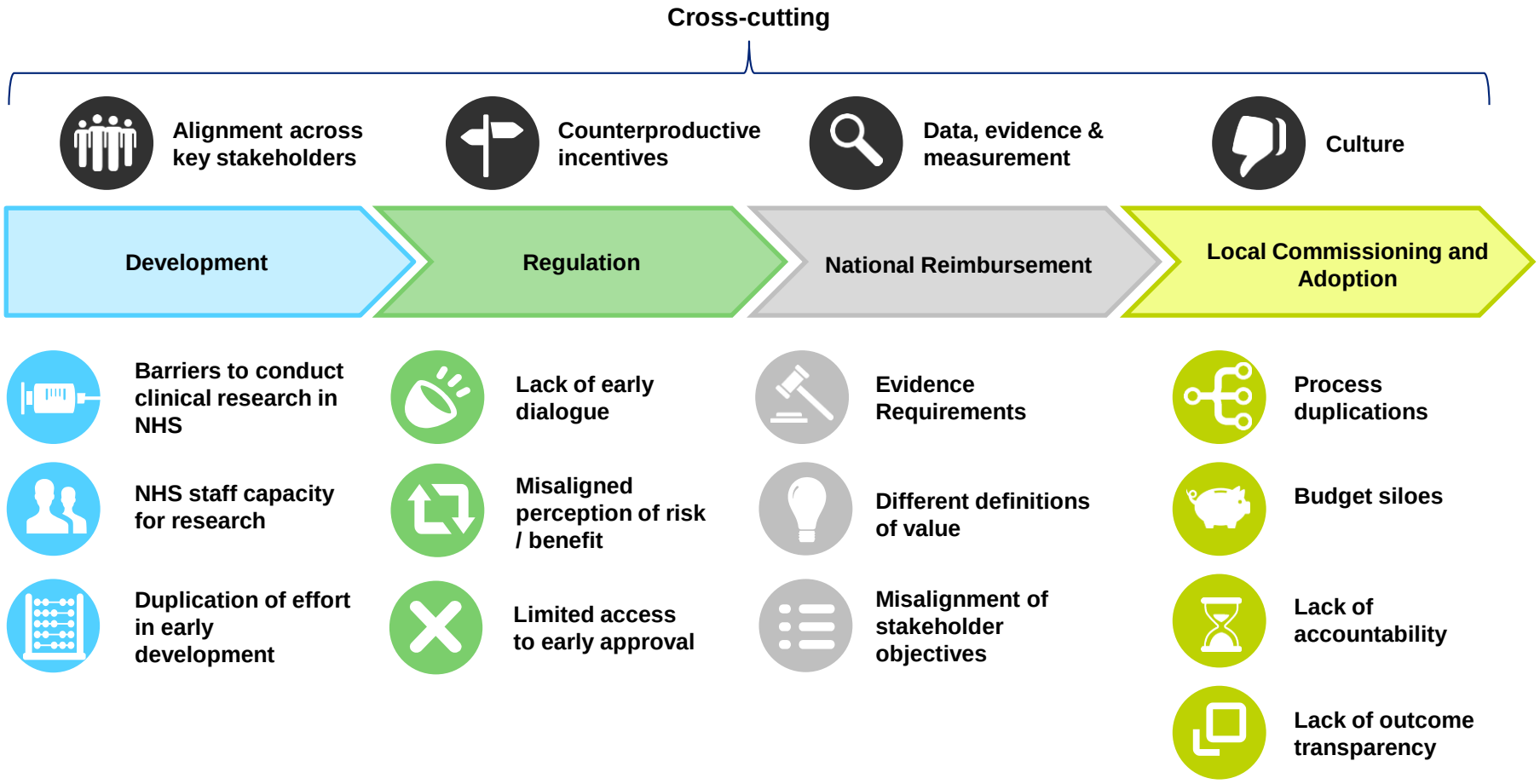
3 Illustrative quotes

4 Stakeholder views of the key challenges

Contents

Challenges: Pharmaceuticals

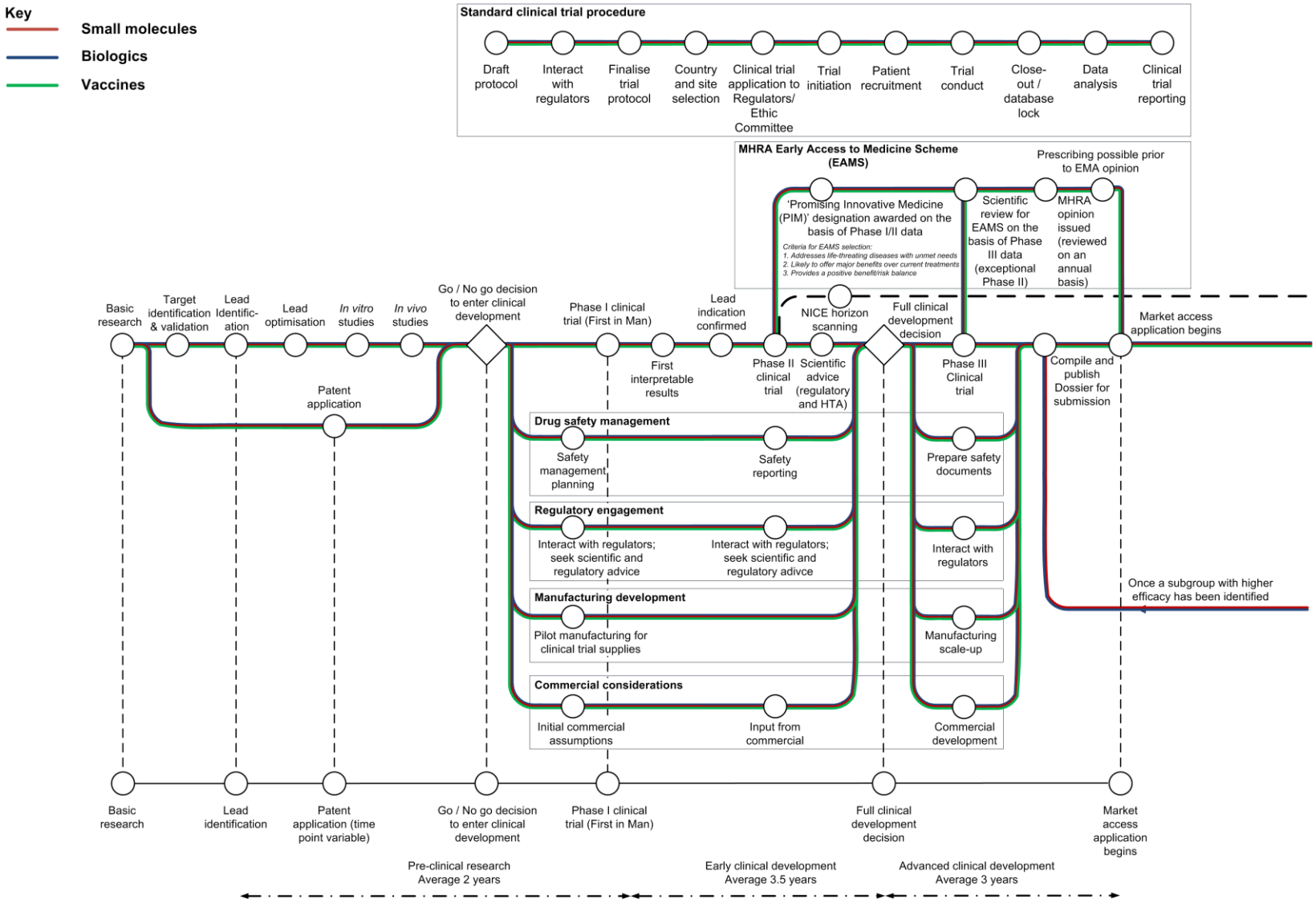
Our research also highlighted a number of specific challenges to pharma by pathway stage



 Source: Stakeholder interviews, workshops and Deloitte analysis

Pharma: Development

Development: *Detailed Pathway*



Source: Stakeholder interviews, workshops and Deloitte analysis

Development: *Key Challenges*

Our research highlighted three key challenges in the development stage for innovative medicines

Barriers to conduct clinical research in NHS

- **Multiple barriers** exist for performing clinical trials in the UK compared to other countries:
  - **Low relative adoption** of innovative medicines (limiting comparator use);
  - **Administrative burden** of trial set up with the NHS; and
  - Poor **data infrastructure**

NHS staff capacity for research

- **NHS staffing constraints** and **few incentives** to engage in clinical research has resulted in a low **availability of research focussed healthcare professionals** to conduct research in the NHS

Duplication of effort in early development

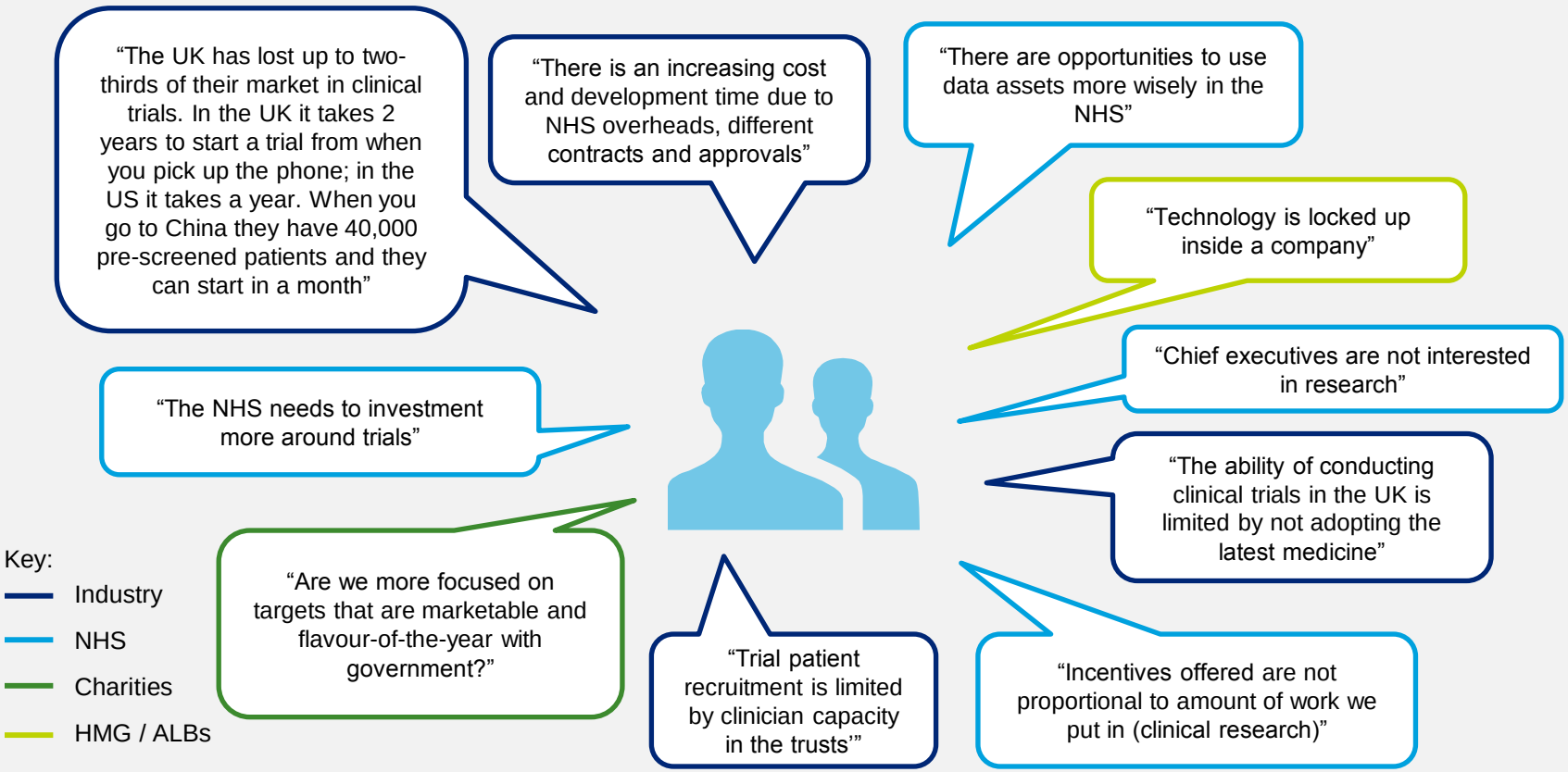
- Considerable **duplication of research effort** exists during early stage development due a **lack of collaboration during the pre-competitive stage**.
  - In particular, interviewees highlighted a lack of Industry-Academia and Industry-Industry collaboration, where **knowledge of success or failure is not shared**, potentially resulting in wasted effort

 Source: Stakeholder interviews, workshops and Deloitte analysis

Development: *Illustrative Quotes*

Quotes summarise some of the key challenges in the development stage for innovative medicines

COMMENTS ON DEVELOPMENT



Source: Stakeholder interviews, workshops and Deloitte analysis

Development: *Stakeholder views*

INDUSTRY VIEWS

- Consensus among interviewed stakeholders that **UK has a strong capability** to conduct medical research and development
- There is a **lack of incentives** and recognition for clinicians and nurses to **work on clinical trials**
- Interviewees mentioned **increasing cost and efforts** associated with setting up clinical trials in the NHS with **duplicated contract negotiation** and **stakeholder engagement** in each trust
- **Misalignment of commercial interests and risks** between stakeholders resulting in loss of research value; value is further lost via duplicating development efforts with different stakeholders

HMG / ALBs VIEWS

- **Industry is reluctant to engage NICE** and others on product development and evidence collection at an early stage
- **Fees are a potential barrier** to early stage companies in seeking advice, particularly for SMEs
- **Knowledge and technologies** are **not shared between Industry and other organisations** during the pre-competitive stage to enable the risk of early development to be better shared

NHS VIEWS

- The NHS interviewees mentioned a **lack of specialised R&D nurses**, **clinician capacity and time** for conducting trials
- The **level of revenue** generated by taking part in trials **doesn't reflect the workload** required
- Payment is based on recruitment number, **not the complexity** of the study
- There is **misalignment between local and global office of study sponsor** leading to multiple negotiation processes
- NHS data are robust and can support licensing, but there is a **poor data management**

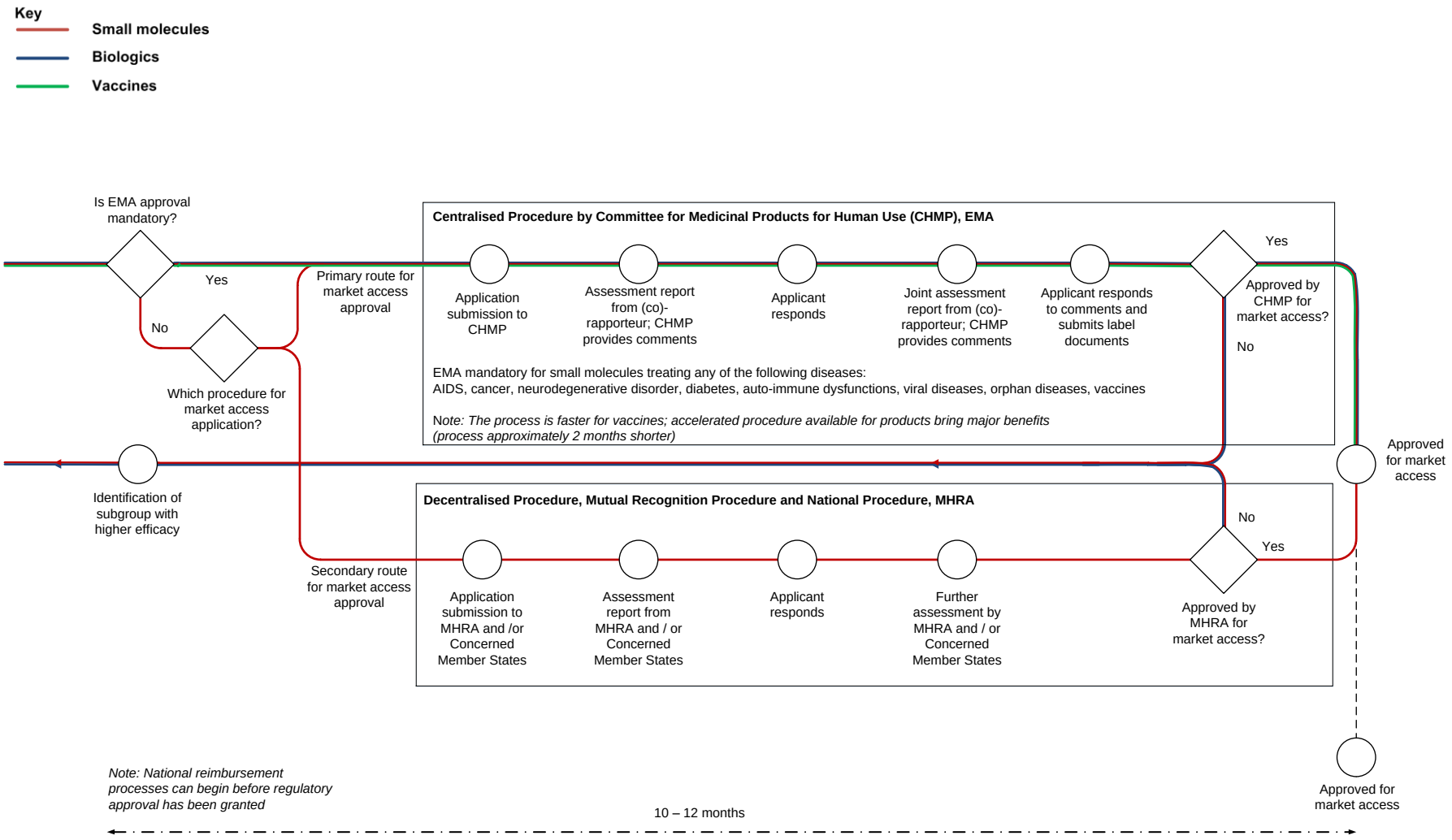
CHARITY VIEWS

- Patient groups feel like that the **current development process is too long** and the development paradigm **does not work for rare and highly stratified** molecularly targeted therapies
- Concerns that the **industry cannot invest without reasonable prospect of returns**, especially for small patient populations

 Source: Stakeholder interviews, workshops and Deloitte analysis

Pharma: Regulation

Regulation: *Detailed Pathway*



Source: Stakeholder interviews, workshops and Deloitte analysis

Regulation: *Key Challenges*

Our research highlighted three key challenges in the regulatory stage for innovative medicines

Lack of early dialogue

- **Selective uptake by Industry of available advice** on protocol and trial design and low update of HTA early dialogue:
  - for SMEs **price** is a concern;
  - big pharma feel they already have **sufficient in-house capability**

Misaligned perception of risk / benefit

- Different **perception of risk and benefit balance** across different stakeholder groups; for example **very high unmet need** patient groups are **willing to adopt earlier**, but current systems are **unable to take these scenarios into account**

Limited access to early approval

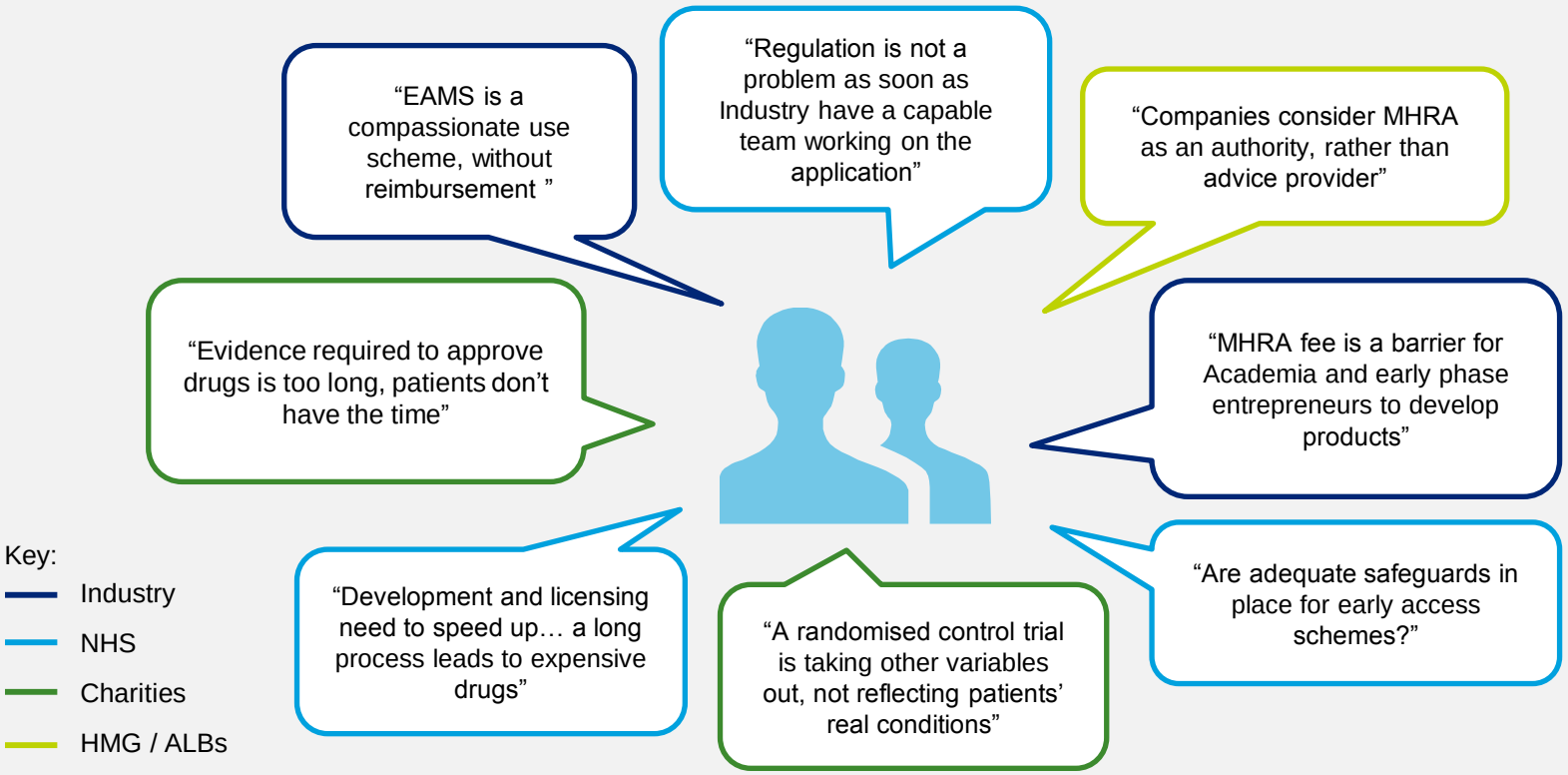
- Although becoming more common, **conditional regulatory approval** on limited (Phase I / Phase II) clinical data sets based on **continued demonstration of safety** and efficacy is **restricted to certain product types**.
  - This reduces the speed of access to regulatory approval

 Source: Stakeholder interviews, workshops and Deloitte analysis

Regulation: *Illustrative Quotes*

Quotes summarise some of the key challenges in the regulatory stage for innovative medicines

COMMENTS ON REGULATION



Source: Stakeholder interviews, workshops and Deloitte analysis

Regulation: *Stakeholder Views*

INDUSTRY VIEWS

- There is a **high barrier for receiving conditional approval** from the regulatory authorities
- Industry stakeholders are **hesitant to engage with adaptive pathway schemes**, since there is a lack of reimbursement and a clear adoption plan at the end of scheme
- On early dialogue interviewees have expressed that:
  - SMEs consider the **service costly** and unnecessary as they do not intend to seek licensing directly
  - Large pharma consider that they are able to assess regulatory requirements with **internal capabilities**

HMG / ALB VIEWS

- **Industry is not engaging with regulatory bodies for scientific advice** around data requirements, especially during earlier stages of development
- **Agile pathways and fast track processes** are available, but only for medicines addressing **unmet clinical needs or** offering **major benefits**

NHS VIEWS

- There is a strong opinion from NHS stakeholders that the **licensing process needs to speed up**
- A **few** participants raised **concerns** around whether **adequate safeguards** are in place for **early access** schemes
- Clinicians have commented that the selection criteria for randomised controlled **trials do not represent the common patient populations**

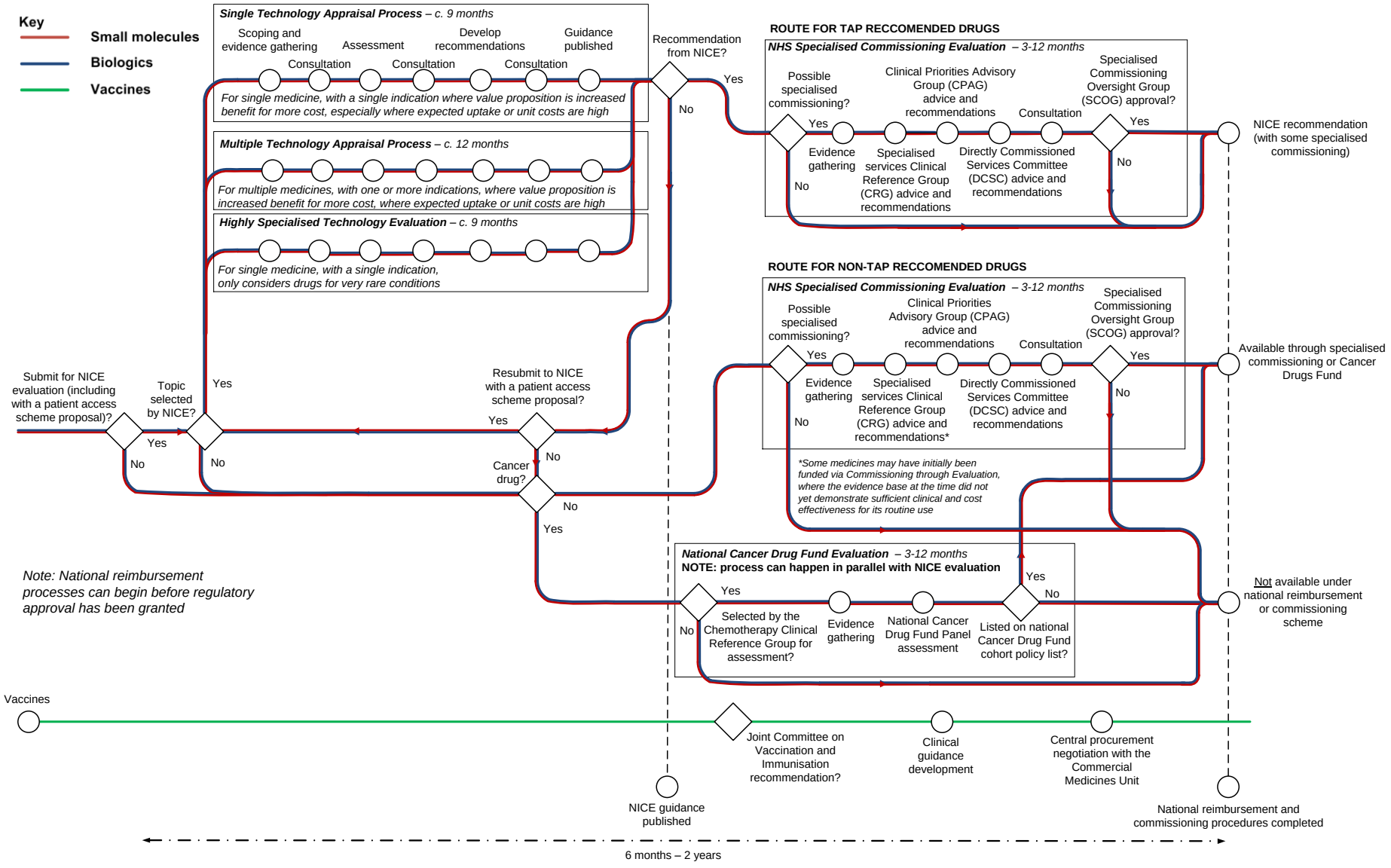
CHARITY VIEWS

- Representatives from patient groups have expressed that patients with **unmet clinical needs** are **willing to accept** innovative treatment at a **higher risk**, but the current **regulation** is **too rigid** for this to happen
- The current **review process is too long**
- A **"one size fit all approach" assessment might not work** for different disease areas

 Source: Stakeholder interviews, workshops and Deloitte analysis

Pharma:
National Reimbursement

National Reimbursement: *Detailed Pathway*



33 Source: Stakeholder interviews, workshops and Deloitte analysis

National Reimbursement: *Key Challenges*

Our research highlighted three key challenges in national reimbursement for early adoption of innovative therapeutic medicines

Evidence requirement

- **Evidence requirements** for pricing and reimbursement are **not typically considered by Industry** during **earlier stages of development** activity (in part due by the global nature of data collection), resulting in **iteration** of the assessment process or **failure to demonstrate sufficient value**

Different definitions of value

- Industry and public sector are **misaligned** on the question of a **drug's value**, e.g..
  - Industry disagrees on the **methodologies applied by NICE** and other HTA bodies to assess the cost-effectiveness and optimal use of medicines;
  - Some parts of public sector consider the **NICE cost per QALY** threshold to be **too high**

Misalignment of stakeholder objectives

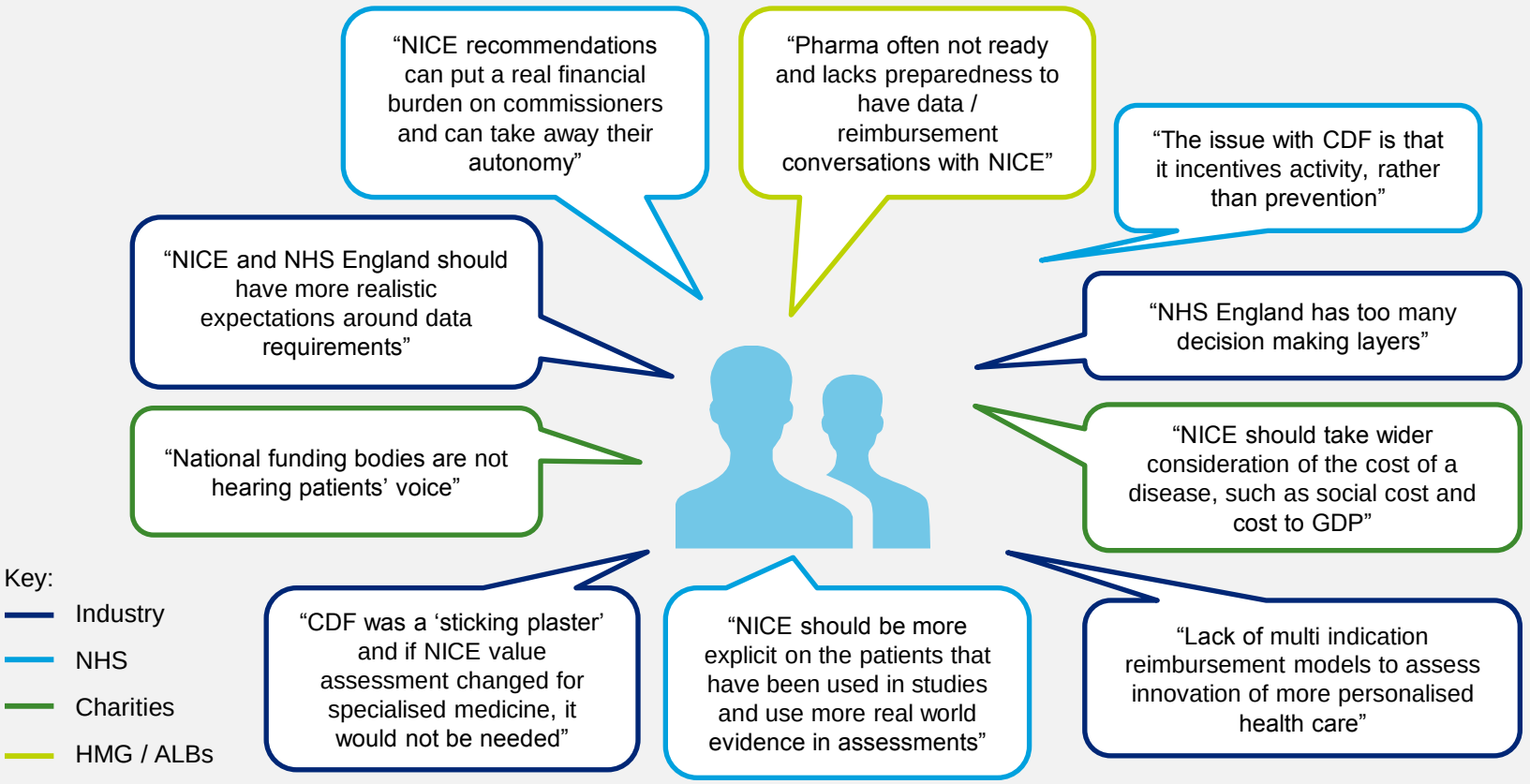
- **Misalignment of objectives** between **NICE and other stakeholders** (e.g. NHS England, patient groups and Industry) over strategic priorities and technology implementation, leading to **low or slow adoption of NICE-recommended medicines**
  - E.g. different priorities for innovation within the organisation mandates of **NICE** (assesses cost-effectiveness), **NHS England** (budget balancing focus) and **Monitor** (provider-level governance compliance)

 Source: Stakeholder interviews, workshops and Deloitte analysis

National Reimbursement: *Illustrative Quotes*

Quotes summarise some of the key challenges in national reimbursement of innovative therapeutic medicines

COMMENTS ON NATIONAL REIMBURSEMENT



 Source: Stakeholder interviews, workshops and Deloitte analysis

National Reimbursement: *Stakeholder Views*

INDUSTRY VIEWS

- Industrial stakeholders agreed that there is a misalign**ment of data requirement** between NICE and regulatory bodies
- The **current assessment by NICE and NHS England** does not support **flexible pricing or personalised medicine**
- **NICE's methodologies** are considered **too academic**, not taking full account of the value of medicines and the **assessment logic is not used for other areas of NHS** (e.g. A&E, single-sex wards)
- **Decision-makin**g process for **specialised medicine** in NHS England is **not transparent**

HMG / ALB VIEWS

- There is **no alignment between NICE and NHS England** on prioritising or highlighting **unmet clinical needs**, making NICE's selection process difficult
- **Companies are not engaging early** for advice on evidence requirement from NICE

NHS VIEWS

- Interviewees from the NHS indicated that although **NICE assessment is comprehensive**, it is **standardised and may not reflect local needs**
- There was a feeling from **some** participants that NICE should ensure assessments **make it easier for commissioners to adopt** (i.e. more information on patients that have been used in studies, more real world evidence use)
- **Some** interviewees felt that **NICE recommendations** can put **financial burdens on commissioners** and reduce their autonomy

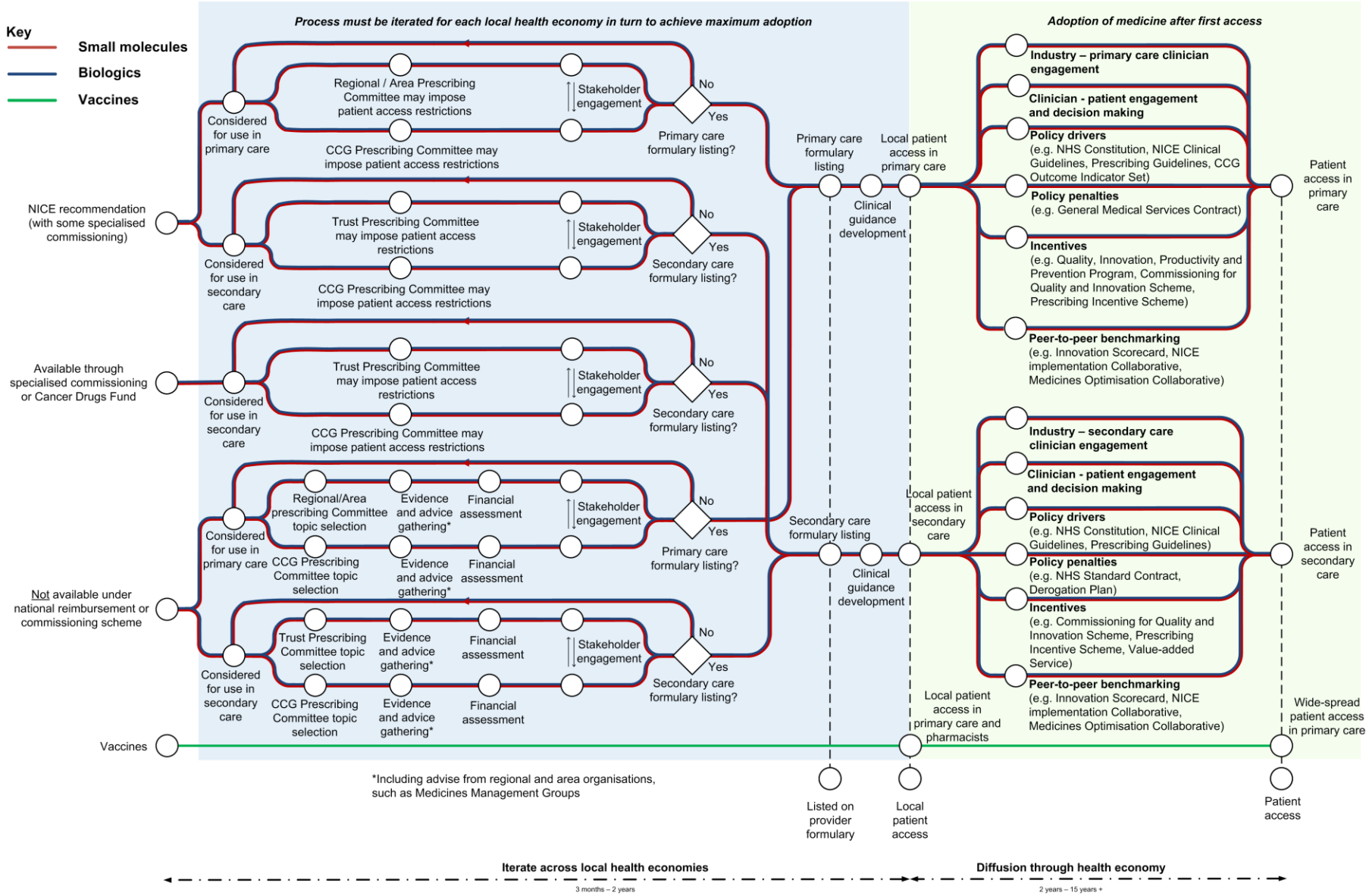
CHARITY VIEWS

- NICE assessment is **not considering the overall cost effectiveness to patient and society**
- NICE and NHS England selection as seen not drawing attention to **small patient group treatment**

 Source: Stakeholder interviews, workshops and Deloitte analysis

Pharma:
Local Commissioning & Adoption

Local commissioning & adoption: *Detailed Pathway*



 Source: Stakeholder interviews, workshops and Deloitte analysis

Key Challenges

Our research highlighted four key challenges in local commissioning for early adoption of innovative therapeutic medicines

Process duplication

- There is **significant duplication of effort**, with **multiple bodies** conducting similar reviews of the same data. The **lack of central coordination** results in **no clear route to market r**equiring costly sales process duplication to address all CCGs / providers for Industry

Budget Siloes

- **Internal budget siloes** between NHS organisations and within departments of an organisation **disincentivizes adoption where cost and value realised are not aligned**
  - In addition **costs and benefits accruing in different budgeting time periods** further limits adoption as effective mechanisms of **funding over a longer timeframe** are not available

Lack of accountability

- **Local adoption** of innovative medicines can be **slow / hesitant** with **limited decision-making accountability to national policy**, even for NICE-recommended or specialised commissioning medicines

Lack of outcome transparency

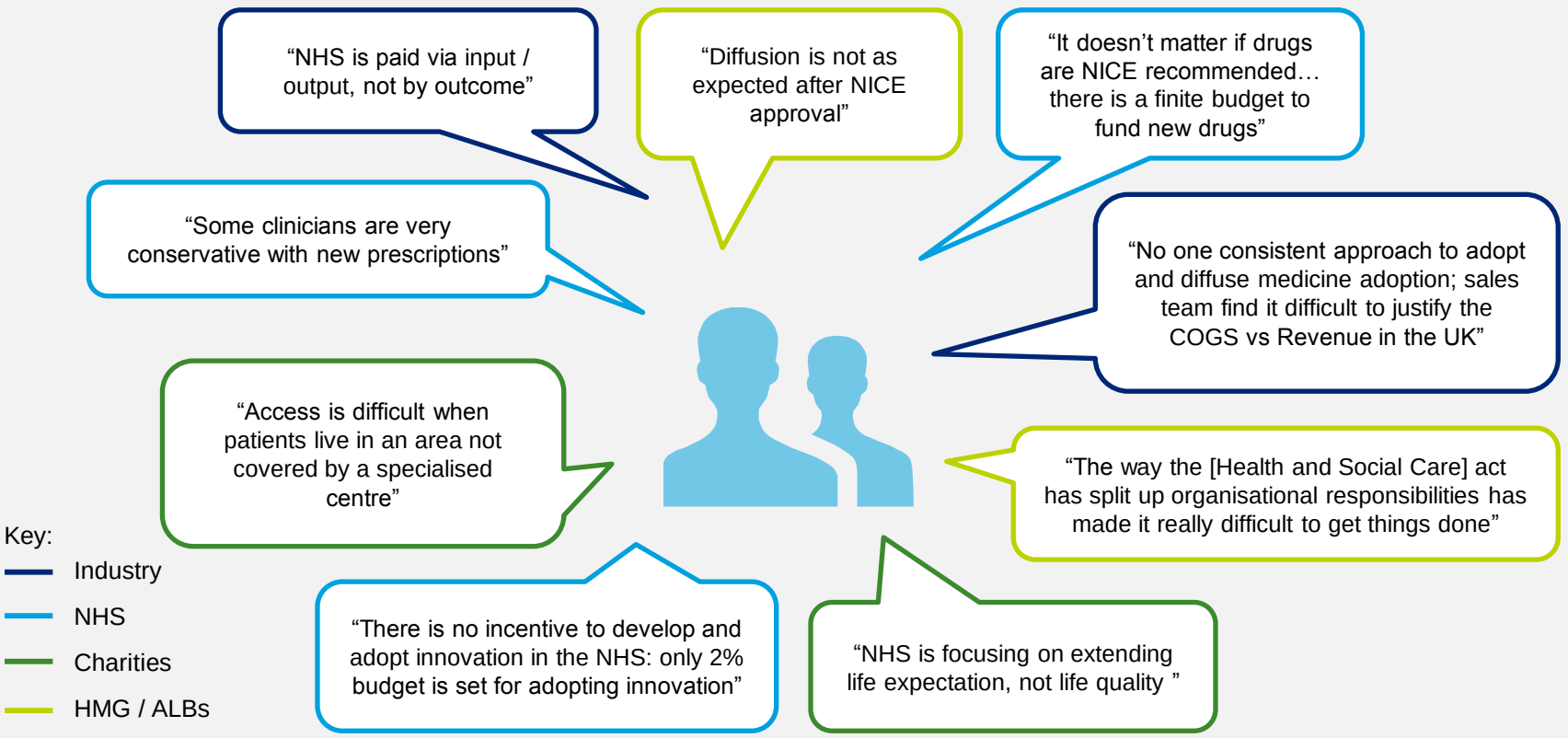
- **Real world effectiveness is not standardly captured or reported**, this hinders the **creation of an evidence base** that could be used to drive adoption of medicines that are seen to be cost-effective in practice.
  - **Few or no incentives for decommissioning of products**
  - Complicated and **poorly understood local decision-making** processes

 Source: Stakeholder interviews, workshops and Deloitte analysis

Local commissioning & adoption: *Illustrative Quotes*

Quotes summarise some of the key challenges in national reimbursement of innovative therapeutic medicines

COMMENTS ON LOCAL COMMISSIONING AND ADOPTION



 Source: Stakeholder interviews, workshops and Deloitte analysis

Local commissioning & adoption: *Stakeholder Views*

INDUSTRY VIEWS

- Industrial stakeholders emphasised that there are **duplicated assessments** of the same medicine **across CCGs and providers**, causing delays and **variation of adoption**
- There is a **lack of budget to adopt** innovative medicines and **lack of flexibility to system change** despite clear evidence of good benefit / cost balance
- Industrial stakeholders also agreed there is **misalignment of incentives** to promote adoption and **few penalties** if they don't

HMG / ALB VIEWS

- **NICE and NHS England's decisions** are **not been carried out** by local providers and **90 days formulary** listing rule is used as an **excuse to delay adoption**
- Interviews with ALB representatives indicated that **providers are not assessed based on their adoption of innovation**

NHS VIEWS

- It is **difficult to adopt new products** especially those **requiring system redesign** e.g. through double-running cost of old and new adding a burden to the medicine budget
- It is **challenging to bring providers together** and to **balance different incentives and benefits** for pathway redesign
- NHS stakeholders interviewed indicated that **new medicines are often too expensive** and there is a lack of funding for adoption
- Individual clinicians have different view on drugs and it is **difficult to change their prescription behaviours**

CHARITY VIEWS

- NHS commissioners are not **focusing on quality of life**, but on **meeting performance standards** and **financial savings**
- Many patient groups mentioned that **some specialised drugs are only available in selected providers**, resulting in a longer delay for patients to access these treatments in some areas
- There are also concerns that Industry **is only focusing on getting reimbursement**, not on what clinicians or patients need

 Source: Stakeholder interviews, workshops and Deloitte analysis

Pharma:
Case Studies

Case study: Pharma

The widespread adoption of a new class of drugs for preventing stroke is limited despite clinical and cost-effective benefits

Background

- Non-Vitamin K antagonist oral anticoagulants (NOACs) include dabigatran, rivaroxaban and apixaban were approved by NICE in 2014 as options for stroke prevention
- They were recommended as first-line therapy over traditional treatments (e.g., warfarin & aspirin) and demonstrated improvements in both clinical efficacy and cardiac risk management
- NOACs costs are significantly higher than traditional treatments
- However NICE has concluded that the drugs are cost-effective and must be available to patients for on-label use
- The EHRA, NICE and European Society of Cardiology have all issued separated guidelines recommending NOACs

Issues

- Despite the NICE recommendation, adoption has been limited
- Clinicians believe in the superior safety of aspirin which was communicated in NICE 2006 guideline while some remain unaware of the development of novel medicines in the recent years
- Moreover, primary care practitioners often do not want to take responsibility for the first prescription of a NOAC, preferring instead to refer patients to secondary care anticoagulant clinics, which increases system costs
- Data from Quality and Outcomes Framework, which details GP practice performance, indicates that only 65% of eligible patients in England received NOACs between 2012 – 2013

Challenges

Alignment across key stakeholders

Culture

Budget Siloes

Lack of accountability

Lack of outcome transparency

Note: EHRA = European Heart Rhythm Association
Source: NICE, EHRA, European Society of Cardiology, Deloitte interviews

Contents

Challenges: Medical devices

Our research also highlighted a number of specific and different challenges to medical devices by pathway stage

Cross-cutting

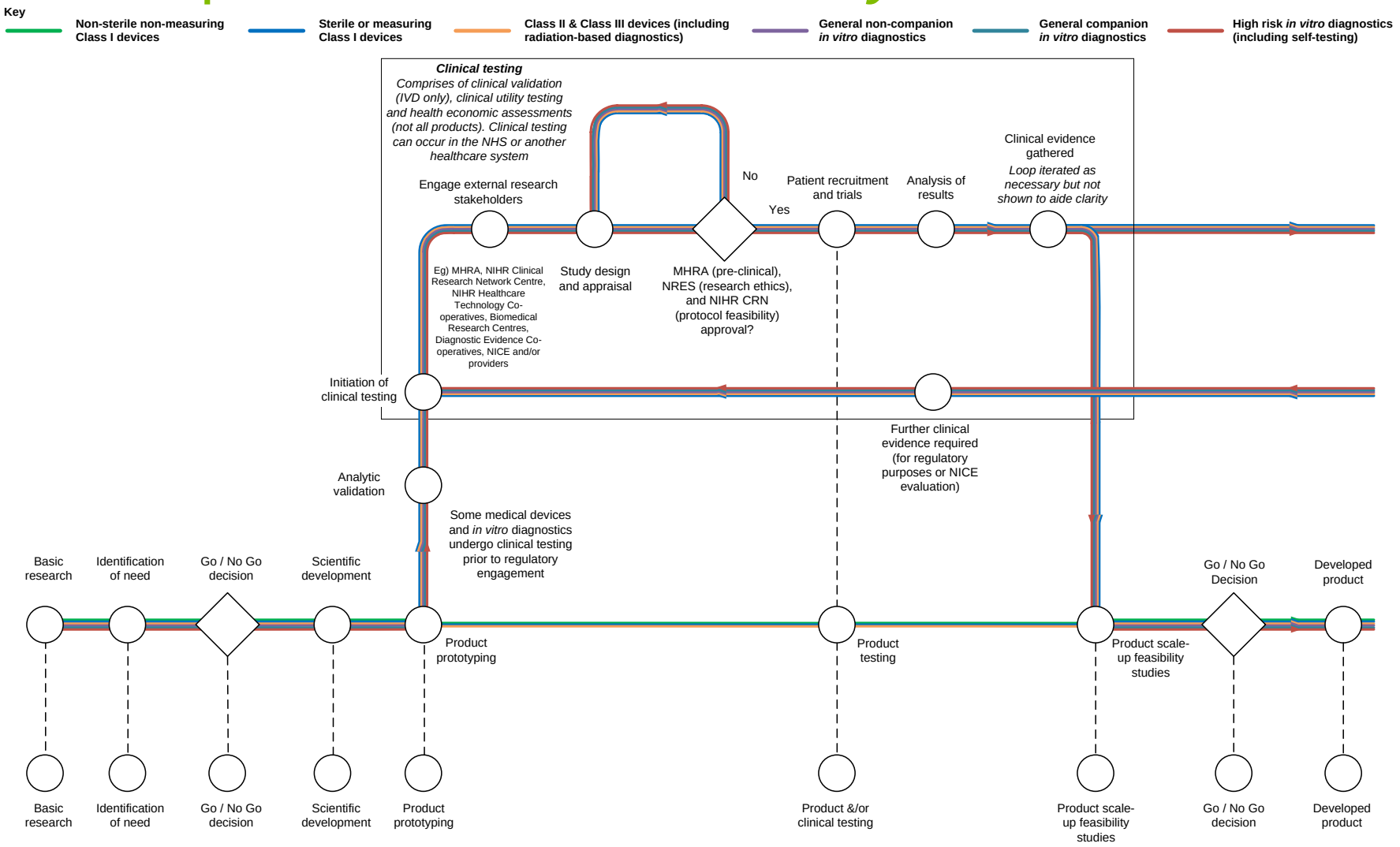
- **Alignment across key stakeholders**
- **Counterproductive incentives**
- **Data, evidence & measurement**
- **Culture**

Development	Regulation	National Reimbursement	Local Commissioning and Adoption
Lack of clinical & commercial capability	Lack of early dialogue	Lack of funding mandate	Unclear route to market
NHS trial approvals process	Low barriers to entry creating later adoption problems	Lack of commercialisation capability	Process duplication
		NICE turnaround times	Budget siloes
		System requires Industry push	Speed of pricing / contracting update
			SME expertise / scale

 Source: Stakeholder interviews, workshops and Deloitte analysis

Medical devices: Development

Development: *Detailed Pathway*



 Source: Stakeholder interviews, workshops and Deloitte analysis

Key Challenges

Our research highlighted two key challenges in the development stage for innovative medical devices

Lack of clinical & commercial capability

- **Some concerns** that there can be a **focus within development**, particularly for SMEs, on **solving engineering challenges** rather than **identifying a clinical need** and generating evidence of clinical benefit and **commercialisation**, leading to adoption problems downstream

NHS trial approvals process

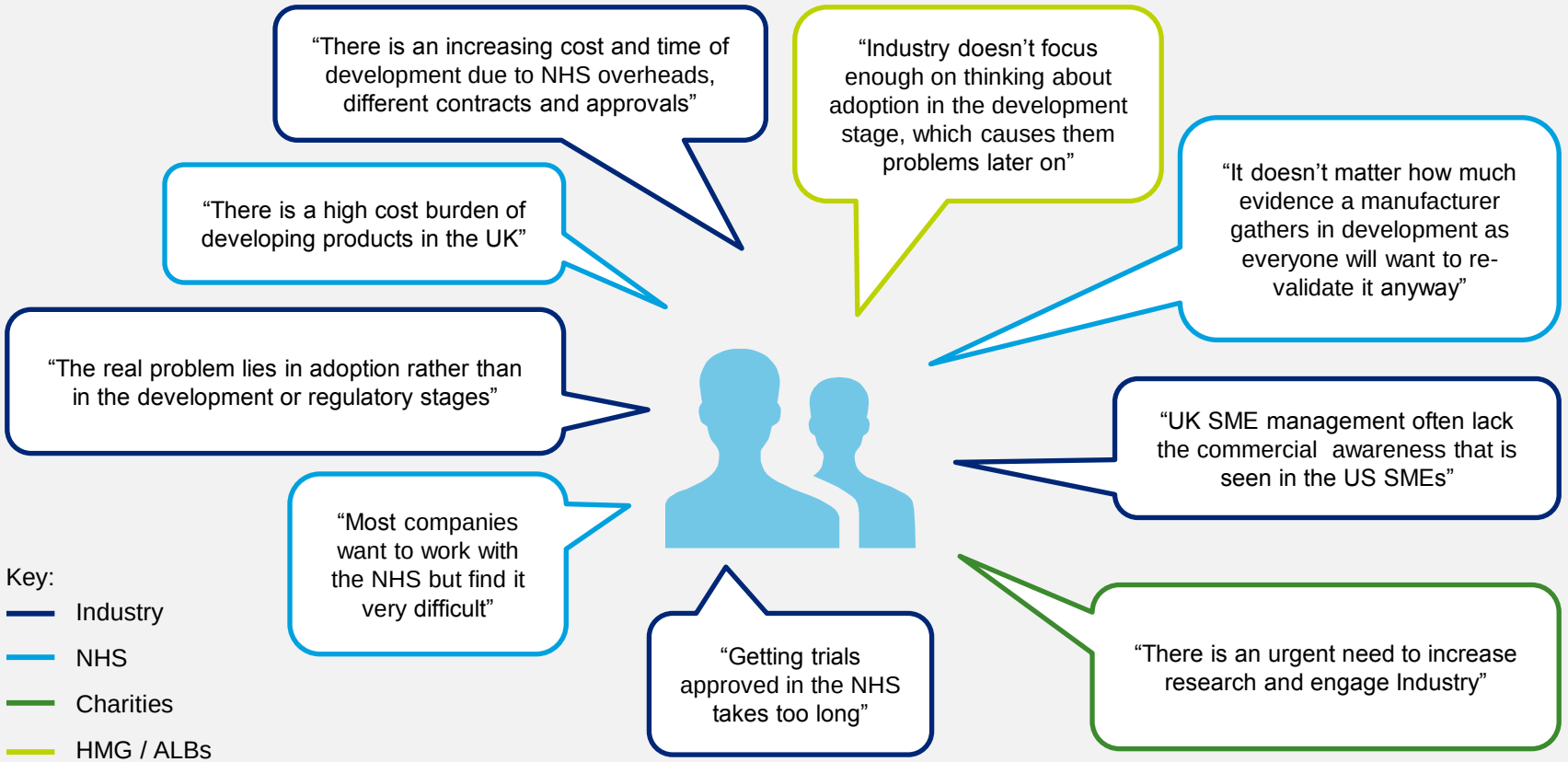
- **Slow trial approval processes** (e.g., ethics and data / sample consenting) raise **costs of clinical evidence collection** (especially for SMEs), delaying adoption

 Source: Stakeholder interviews, workshops and Deloitte analysis

Development: *Illustrative Quotes*

Quotes summarise some of the key challenges in the development stage for innovative medical devices

COMMENTS ON DEVELOPMENT



 Source: Stakeholder interviews, workshops and Deloitte analysis

Stakeholder Views

INDUSTRY VIEWS

- Most Industry stakeholders believe the **NHS trials approvals process is too time consuming**
- The **cost of clinical trials** is a **burden for SMEs,** particularly in developing clinical evidence
- Industry feel that there are **limited incentives** for Trusts and healthcare professionals to get **involved with clinical trials**
- **Successful** companies **focus** on **generating evidence of clinical benefit** and are clear about their **route to adoption**
- A **few** stakeholders think **grant funding** should be directed to medical devices that have a **high chance of being adopted**

HMG / ALBs VIEWS

- HMG / ALBs are of the view that **Industry do not focus** enough time and resources thinking about their **route to market** in the development stage, which leads to **adoption problems later**
- HMG / ALBs think that **companies do not** always **see the value** of **early dialogue** conversations, especially around study design and evidence requirements
- **Fees are a barrier,** particularly for SMEs to engage earlier with HMG / ALBs
- Sense that **commercialisation capability** is a problem, especially for **SMEs**

NHS VIEWS

- There is a feeling that it **does not matter how much evidence** manufacturers gather in the development stage as **local commissioners and providers** will want to **re-validate it locally**
- Generally Industry **focus on solving the engineering problems** rather than generating **evidence of clinical benefit**
- Most companies want to work with the NHS but find it **hard to partner with the NHS**
- **Staff capacity limits** the **NHS's ability** to work in the development of innovative medical devices

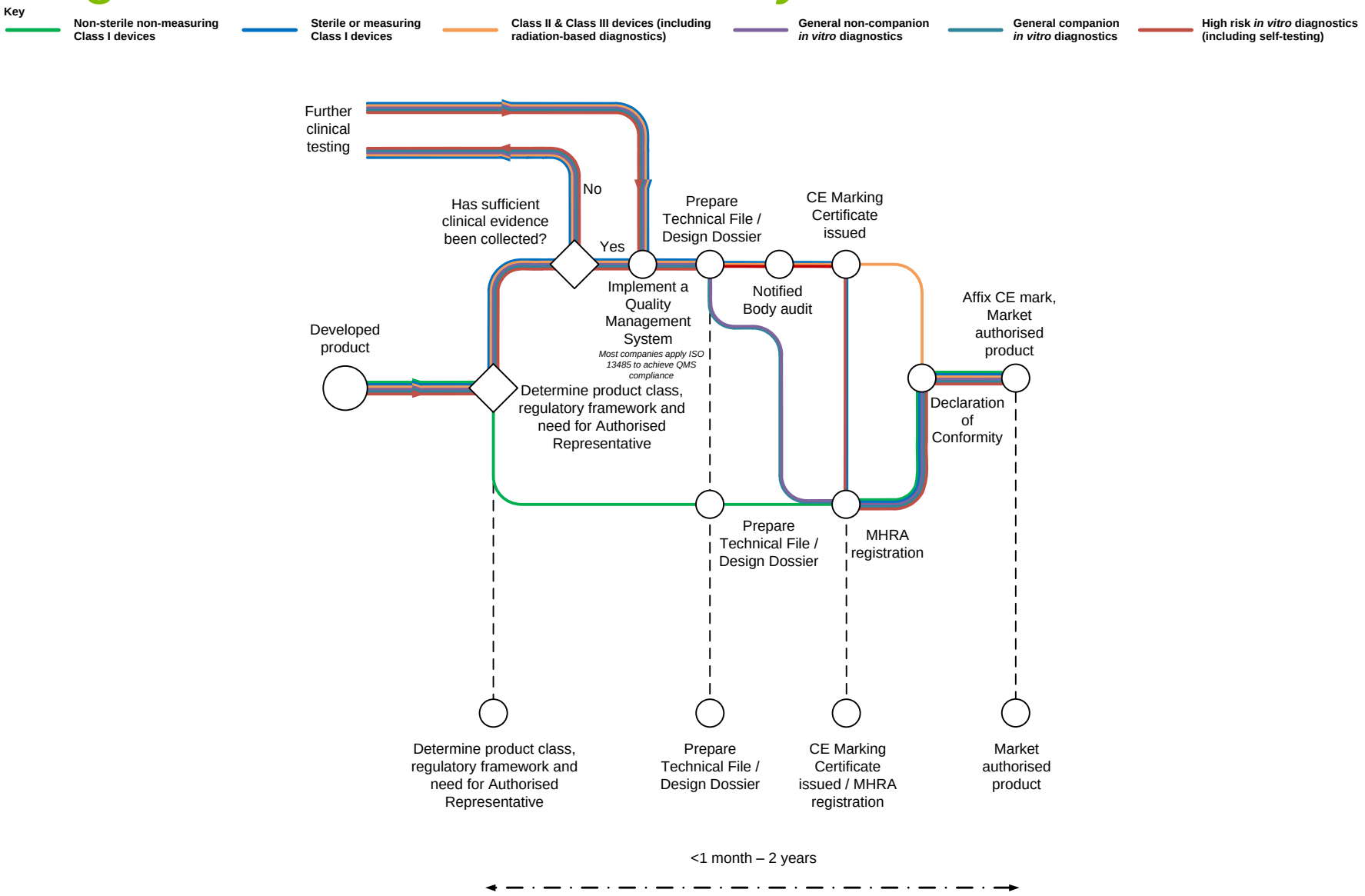
CHARITY VIEWS

- Charities feel that there is an urgent need to **increase research and engage Industry**, especially for disease areas where there has been no or limited treatment or care advances in the last decade
- The NHS should **encourage more patients and clinicians** to get **involved** with **clinical research**
- **Specific diseases** could be used as a **'test-bed' for reform** from development through to adoption
- The review should **build on** what is **already working**

 Source: Stakeholder interviews, workshops and Deloitte analysis

Medical devices: Regulation

Regulation: *Detailed Pathway*



 Source: Stakeholder interviews, workshops and Deloitte analysis

Key Challenges

Our research highlighted two key challenges in the regulatory stage for innovative medical devices

Lack of early dialogue

- **Low uptake of available advice** on protocol and trial design
  - **Not seen as value-add** for companies with deep experience
  - Seen as **too large a time and cost investment** for SMEs
  - Lack of engagement and **uncertainty about effectiveness** of this dialogue reduces partnering

Low barriers to entry creating later adoption problems

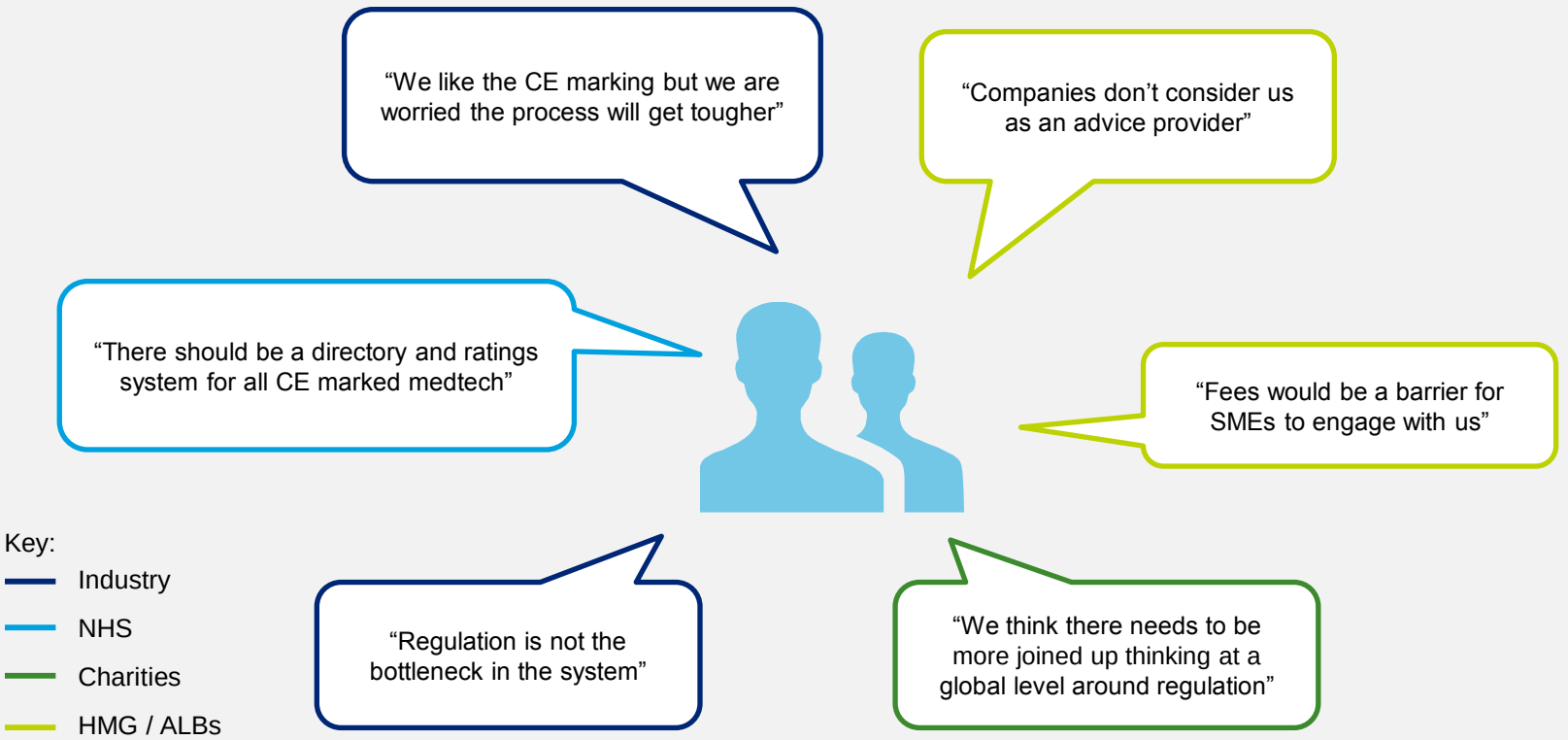
- The **relative ease in which companies are able to CE mark** medical devices, compared to successfully **navigating reimbursement and commissioning** procedures, results in later adoption problems. Too often **studies in the development stage**, particularly those carried out by SMEs, are **not adequate** for national or local **reimbursement and / or commissioning** purposes

 Source: Stakeholder interviews, workshops and Deloitte analysis

Regulation: *Illustrative Quotes*

Quotes summarise some of the key challenges in the regulatory stage for innovative medical devices

COMMENTS ON REGULATION



Source: Stakeholder interviews, workshops and Deloitte analysis

Stakeholder Views

INDUSTRY VIEWS

- Most Industry stakeholders **do not believe regulation is too onerous**
- However, there is a feeling that the lower **evidence requirements for CE marking** compared to reimbursement and commissioning procedures **result in later adoption problems** where studies have focused on **meeting regulatory requirements** rather than reimbursement and commissioning evidence requirements
- **Limited early dialogue** between Industry and HMG / ALBs
- Some Industry stakeholders feel **Notified Body audits are too slow** and that they do not feel engaged with Notified Bodies

HMG / ALBs VIEWS

- HMG / ALBs are of the view that **Industry do not focus** enough time and resources thinking about their **route to market** in the regulatory stage, which leads to **later adoption problems**
- HMG / ALBs think that **companies do not** always **see the value** of early **dialogue** conversations with HMG / ALBs
- **Fees** are a potential **barrier for SME** early engagement
- Feel that Industry generally just considers the **MHRA** as a **regulatory authority**, rather than an **agency** that is **able to provide advice**

NHS VIEWS

- There is the feeling in the NHS that the **regulatory stage** is the stage that is **least challenging**
- There is currently **not a list of all CE marked devices**, which means adoption often **relies on Industry push rather than NHS pull**

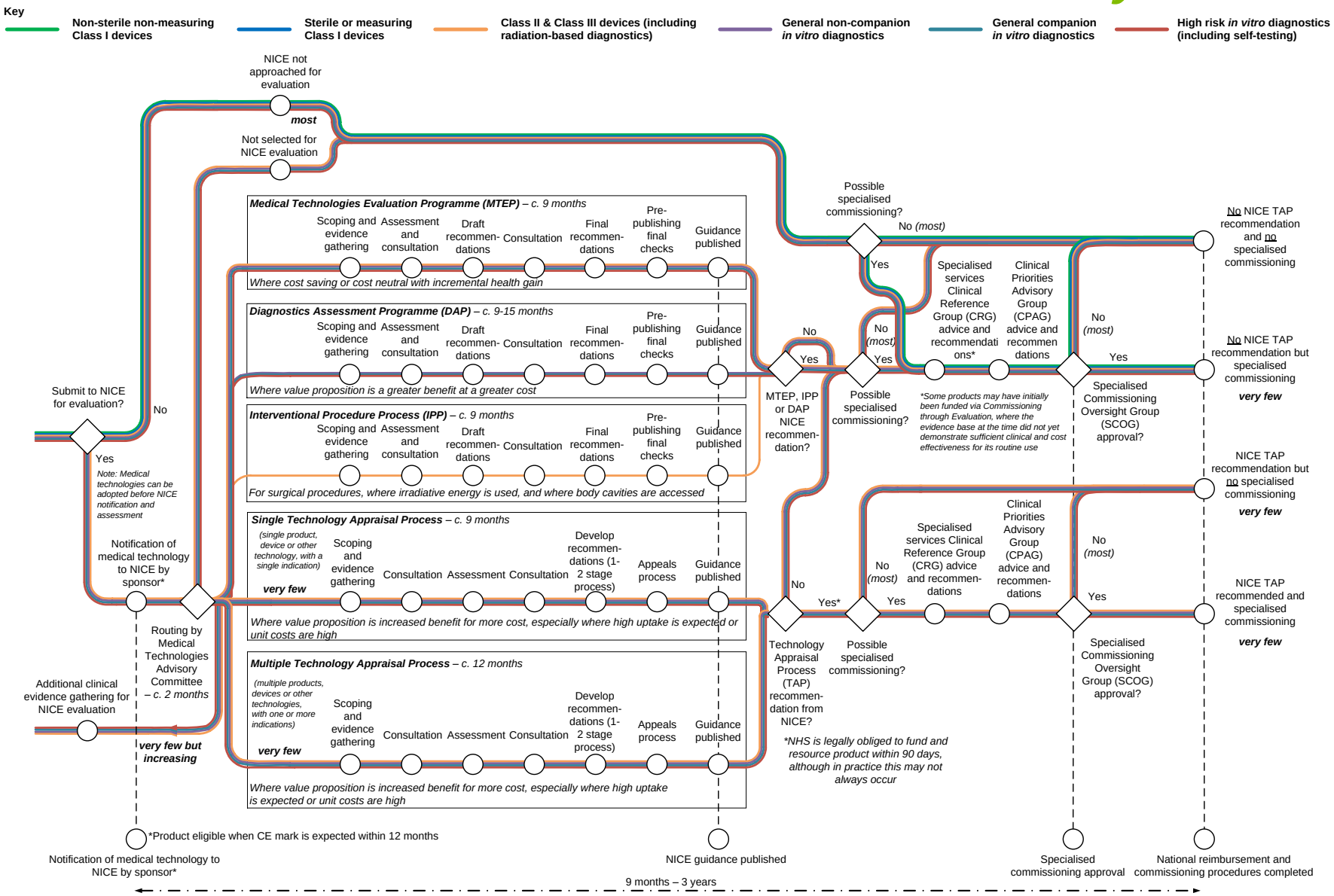
CHARITY VIEWS

- Demand for more **joined up thinking** around **regulation globally**
- **Specific diseases** could be used as a **'test-bed' for reform** from development through to adoption
- The review should **build on** what is **already working**

 Source: Stakeholder interviews, workshops and Deloitte analysis

Medical devices: National Reimbursement

National reimbursement: *Detailed Pathway*



 Source: Stakeholder interviews, workshops and Deloitte analysis

Key Challenges

Our research highlighted four key challenges in the national reimbursement stage for innovative medical devices

Lack of funding mandate

- Only medical devices assessed by the **NICE Technology Appraisal Programme** and found to be cost effective **carry a funding mandate**. Cost-effective **Medical Technology Evaluation Programme (MTEP) recommendations are purely advisory** and do not carry a funding mandate, with limited effect on local adoption
  - The **majority of medical devices are assessed via MTEP**

Lack of commercialisation capability

- Manufacturers (especially SMEs) and healthcare professionals sometimes **struggle to articulate the value proposition** of medical devices, especially using **processes originally designed for pharmaceuticals**

NICE turnaround times

- NICE evaluation **timelines can be too long**, especially when **compared to the innovation cycle of medical devices (often iterative)**, sometimes leading to the **launch of product v2** before NICE has completed an assessment of v1

System requires Industry push

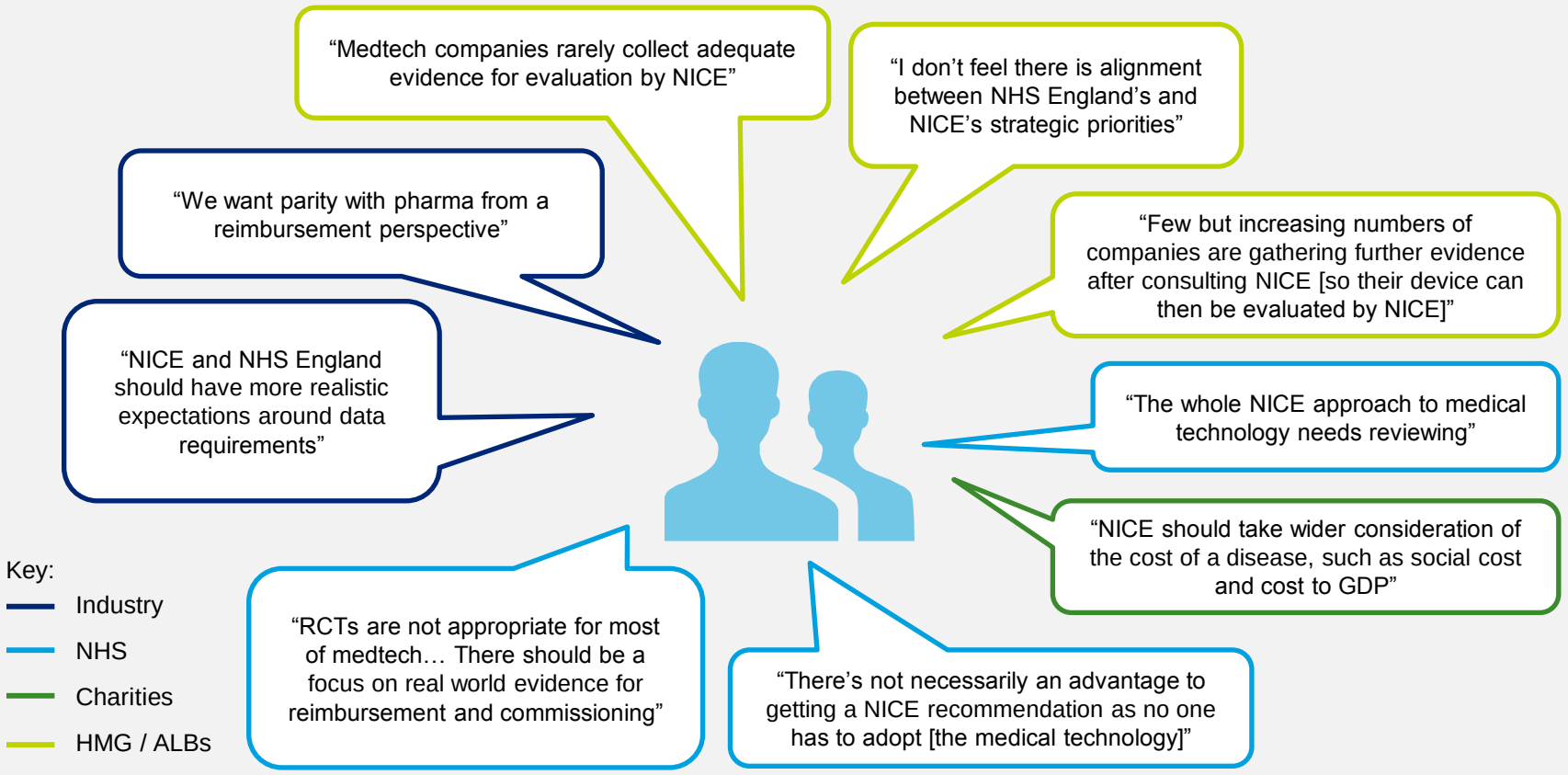
- Suggestion that **Industry** (especially SMEs) are **unwilling to engage with NICE** at early dialogue opportunities or submit products for assessment due to **concerns about success rates,** the **additional cost** and time of gathering sufficient evidence for the NICE assessment process versus the outcome achieved

Note: MTEP = Medical Technologies Evaluation Programme
Source: Stakeholder interviews, workshops and Deloitte analysis

National Reimbursement: *Illustrative Quotes*

Quotes summarise some of the key challenges in the national reimbursement stage for innovative medical devices

COMMENTS ON NATIONAL REIMBURSEMENT



Source: Stakeholder interviews, workshops and Deloitte analysis

Stakeholder Views

INDUSTRY VIEWS

- Industry **would like NICE recommended** medical devices to have a **funding mandate** attached to them
- Feeling that medtech **value propositions can be hard to articulate** in processes that were originally designed for pharma
- Feeling that **NICE turnaround times can be too long**, especially when compared to the innovation cycle
- Suggestion that Industry (especially SMEs) are **unwilling to engage with NICE** at early dialogue opportunities or submit products for assessment due to **concerns about success rates** and the **additional time and cost** of gathering further evidence

HMG / ALBs VIEWS

- There is the general belief that there **could be better alignment between HMG /ALBs** (e.g., between NHS England, NICE and the Department of Health)
- Suggestion that NICE should be having **early dialogue** with Industry in the regulatory and development stages so that the correct advice and **guidance on evidence collection** for national reimbursement and local adoption purposes can be provided
- Companies should be **encouraged to submit to NICE** at the same time that companies begin the CE marking process

NHS VIEWS

- There is the feeling that in the NHS that there is a **disjoint between national and local organisations**; for instance, a recommendation from NICE does not mean that there will be widespread adoption of an *in vitro* diagnostic across CCGs / providers
- There **should be greater use of real world evidence**, rather than RCTs

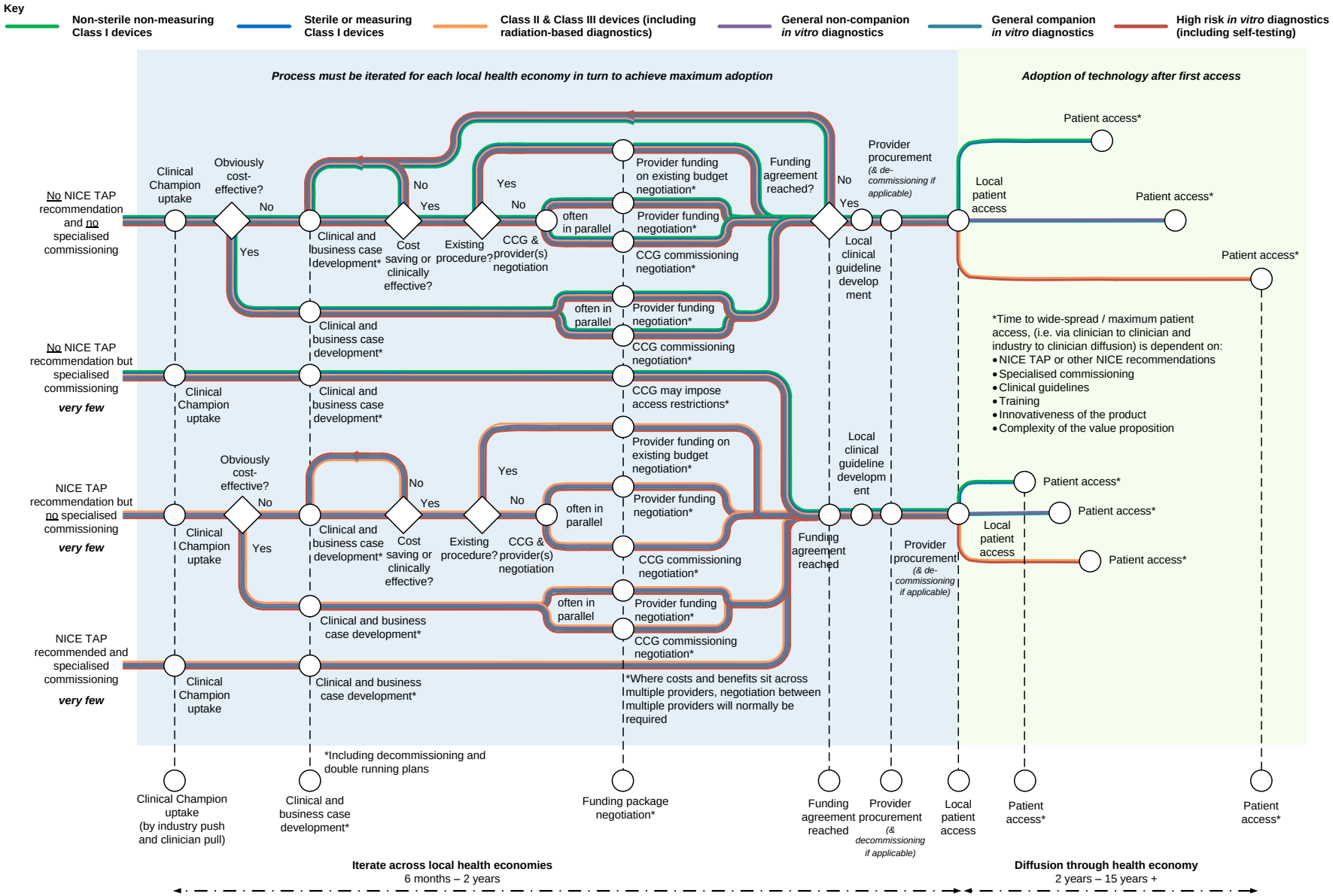
CHARITY VIEWS

- NICE assessment is **not considering** the overall **cost effectiveness** to **patient and society**
- Feeling that NICE **topic selection** does **not focus enough** attention on medtech that would only affect a **small group** of patients
- **Specific diseases** could be used as a **'test-bed' for reform** from development through to adoption
- The review should **build on** what is **already working**

 Source: Stakeholder interviews, workshops and Deloitte analysis

Medical devices: Local Commissioning & Adoption

Local commissioning & adoption: *Detailed Pathway*



 Source: Stakeholder interviews, workshops and Deloitte analysis

Key Challenges

Our research highlighted five key challenges in the local commissioning and adoption stage for innovative medical devices

Unclear route to market

- **No consistent route to local markets** exists for many medical devices, with value **evidence requirements** heavily dependent on **local stakeholder preferences**. There is also a **heavy reliance of personal relationships** to drive adoption

Process duplication

- **Lack of central coordination** and clear route to market results in significant **sales process duplication** across CCGs and / or providers

Budget siloes

- **Internal budget siloes** within and between NHS organisations dis-incentivises the adoption of medical devices where the **costs and value realised are not aligned**
  - **Short budget horizons** and **lost income from lower utilisation** further dis-incentivises investments in medical devices that would **result in savings at an aggregate level**

Speed of pricing / contracting update

- Medical devices often experience **long delays before price and contract update** (e.g., inclusion on Tariff), creating strong dis-incentives for adoption by acute Trusts. Additionally, **large multi-year service contracts** can **stifle competition** and the uptake of innovative medical devices

SME expertise / scale

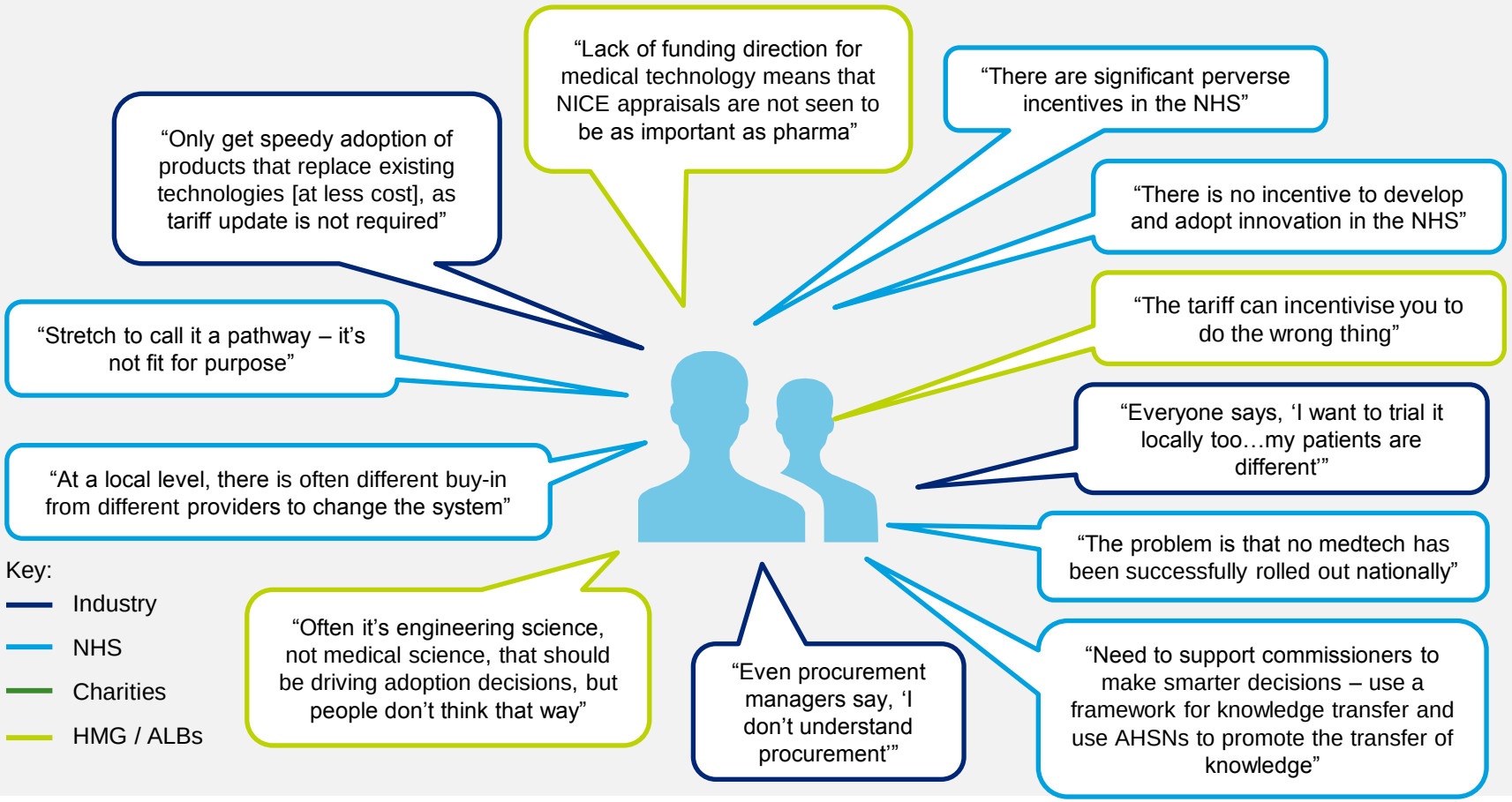
- SME manufacturers report **significant cost-of-sales** issues resulting from the need to **repetitively engage multiple stakeholders** in LHEs across multiple LHEs, as well as uncertainty as to how best to engage

 Source: Stakeholder interviews, workshops and Deloitte analysis

Illustrative Quotes

Quotes summarise some of the key challenges in the local commissioning and adoption stage for innovative medical devices

COMMENTS LOCAL COMMISSIONING AND ADOPTION



Source: Stakeholder interviews, workshops and Deloitte analysis

Stakeholder Views

INDUSTRY VIEWS

- Industry stakeholders interviewed are **strongly of the opinion** that local commissioning and adoption of medtech is the **greatest barrier to patient access**
- **No clear route to market**, with high variability between LHEs and **access highly dependent on clinician relationships**
- **Lack of funding mandate** for non-TAP NICE-assessed products limits adoption
- **Affordability** considered a key barrier to adoption for cost-effective (i.e. not cost-saving) innovations
- Situation especially acute for **SMEs** due to **lack of scale**

HMG / ALBs VIEWS

- Agreement among interviewed stakeholders that **adoption**, especially at local level, is **complex and slow**
- **Lack of a clear route to market** identified as a problem
- Acceptance that **lack of funding mandate** for most NICE-recommended medtech limits local commissioner willingness to pay for innovative medtech
- Feeling that **lack of central coordination** results in significant **duplication** across CCGs and / or providers
- Sense that **commercialisation capability** is a problem, especially for **SMEs**

NHS VIEWS

- **Majority** of interviewed NHS stakeholders believed that local commissioning and adoption is the **greatest barrier to access**
- **Major barriers** to clinician use of medtech are **financial**:
  - **Speed of pricing** and **contracting** update (e.g., inclusion on Tariff) even for NICE-recommended products
  - **Departmental and LHE budget silos** and **horizons** limit the uptake of products, especially where benefits will accrue across multiple care pathways and settings of care
  - **Double running costs** when changes occur
- **Desire to test medtech** on own patients before adopting

CHARITY VIEWS

- Feeling that NHS commissioners **do not focus enough on patients** and their quality of life, and spend too much time **meeting minimum performance standards** and financing constraints
- Feel that some **inequalities between LHEs** are due to **variation** in the **adoption** of innovative medtech
- **Specific diseases** could be used as a **'test-bed' for reform** from development through to adoption

Notes: LHE = Local Health Economy; TAP = Technology Appraisal Programme
Source: Stakeholder interviews, workshops and Deloitte analysis

Medical devices: Case Studies

Case study - Medical devices (1/2)

Adoption of soft tissue support and repair in breast reconstruction surgery is limited by exclusion from National Tariff

Background

- Breast reconstruction is offered to all patients considering a mastectomy
- Based on the results and outcomes of data from the US experience of Soft Tissue Support and Repair (STSR) in breast reconstruction surgery, it is believed that that STSR offers a better patient experience with significant immediate and long-term patient benefits
- In the short term, the appropriate patient would benefit from either a direct reduction in the number of operations required or much reduced theatre time with subsequently reduced morbidity versus other procedures
- In the longer term, US data confirms that downstream complications with STSR are significantly reduced versus a 2-stage approach
- STSR requires the use of implantable medical devices such as acellular dermal matrices or other novel implantable devices

Issues

- Currently there is no specific OPCS code for Soft Tissue Support and Repair (STSR) in breast reconstruction surgery
- Recent attempts at introducing OPCS codes have not been successful
- As a result, funding needs be found from outside the National Tariff on a Trust-by-Trust basis
- Local clinicians have to get funding at a local level by:
    - Securing approval on a trial basis
    - Negotiating for local CCG top-up funding
    - Using existing theatre budget to fund STSRs
- The lengthy and variable process of getting funding for the procedure limits and slows local adoption, while process duplication from Trust-to-Trust limits the widespread adoption of the procedure (and the related medical devices used in the procedure)

Challenges

Alignment across key stakeholders

Counterproductive incentives

Unclear route to market

Process duplication

Budget siloes

Speed of pricing / contracting update

 Source: Stakeholder interviews, workshops and Deloitte analysis

Case study - Medical devices (2/2)

Adoption of cardiac devices with longer battery life is poor because procurement decisions are often made to meet short-term budget constraints

Background

- Cardiac devices such as implantable cardioverter defibrillators (ICD) and cardiac resynchronisation therapy devices with integrated defibrillators (CRT-D) are first line treatments for correcting life-threatening cardiac arrhythmias. These are effective tools to slow the progression of heart failure for at risk patients
- These devices are implanted inside the body and replaced every 4 to 5 years as their battery life deteriorates
- Patients require several device replacements over their lifetimes, which creates additional costs to the healthcare system and increases the risk of complications for patients with each procedure
- Many device manufacturers have improved their battery technology, creating new products with battery lives > 10 years

Issues

- New devices with longer battery lives are more expensive to acquire, but are proven to be cost-effective in the medium term. It is estimated that a 30% cost saving to the system is achieved with the new long-life devices
- Despite the cost-saving and clinical recommendations, many Trusts do not procure the long-life devices as they are not fully reimbursed for them through the national tariff and local top-ups
- Commissioners make decision based on short-term budget constraints
- There are often no / limited incentives to locally adopt products / services that prevent the need for further healthcare interventions, and bring benefits or system savings over the long-term
- Moreover, overall savings for the local health economy from adopting the long-life devices results in a loss of revenue to Trust budgets from a reduction in follow-up procedures

Challenges

Alignment across key stakeholders

Counterproductive incentives

Unclear route to market

Process duplication

Budget siloes

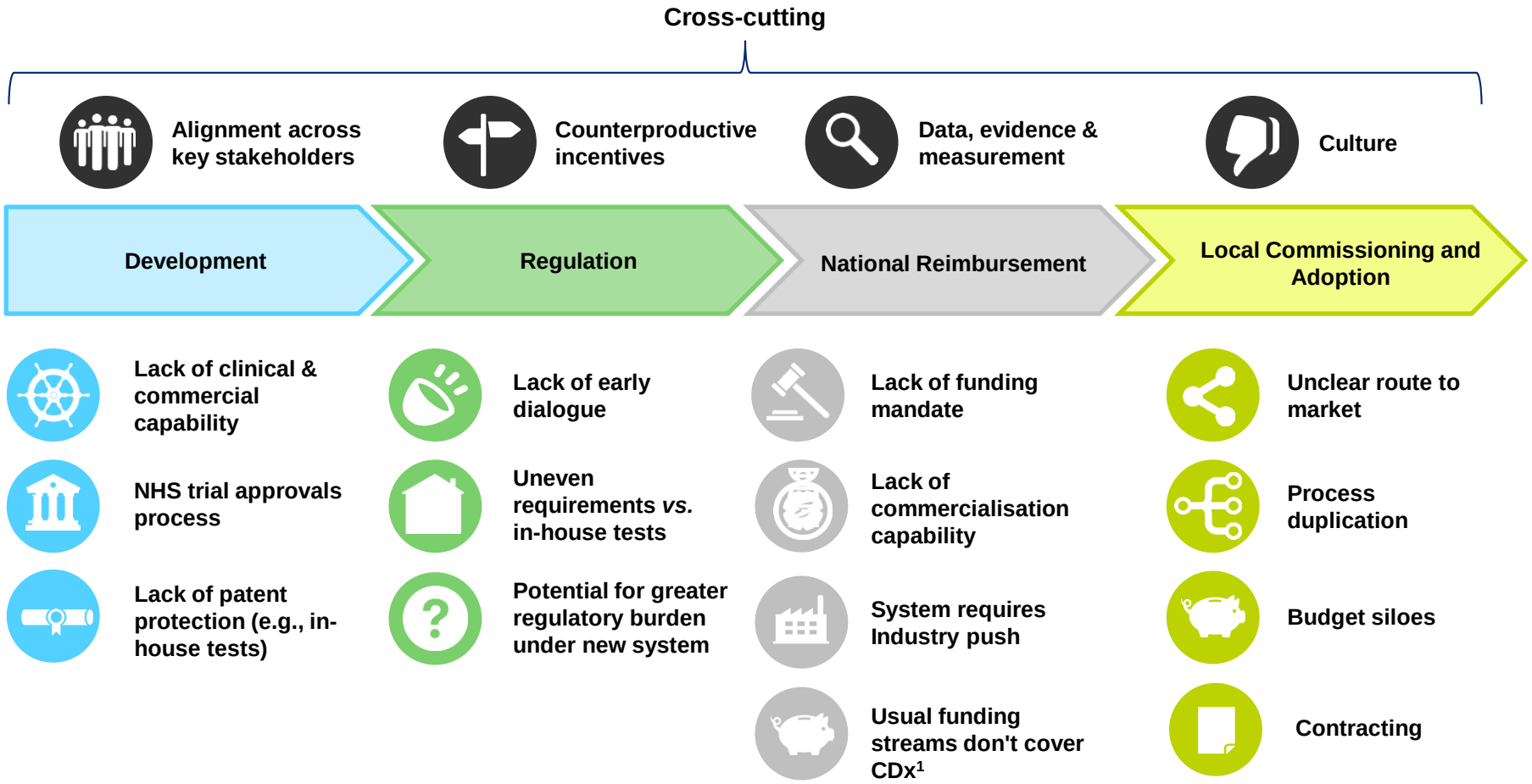
Speed of pricing / contracting update

 Source: Stakeholder interviews, workshops and Deloitte analysis

Contents

Challenges: *In vitro* diagnostics

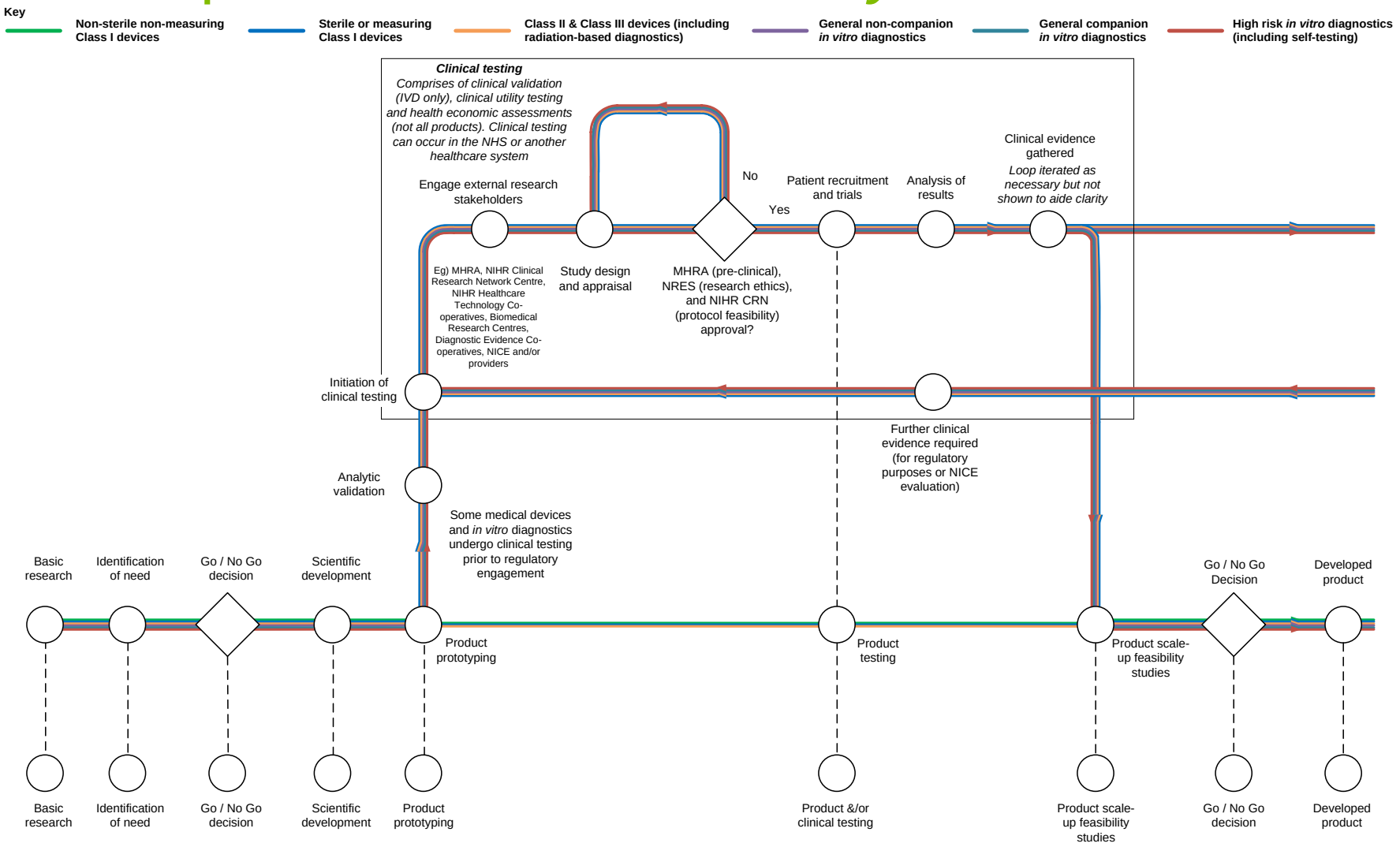
Our research also highlighted a number of challenges that are specific to *in vitro* diagnostics by pathway stage



Notes: (1) CDx = co-diagnostics; Source: Stakeholder interviews, workshops and Deloitte analysis

In vitro Diagnostics: Development

Development: *Detailed Pathway*



 Source: Stakeholder interviews, workshops and Deloitte analysis

Key Challenges

Our research highlighted three key challenges in development for the early access to innovative *in vitro* diagnostics

Lack of clinical & commercial capability

- **Some concerns** that there can be a **focus within development**, particularly for SMEs, on **solving the scientific diagnostic marker challenge** rather than **identifying a clinical need** and focusing on generating **evidence of clinical benefit** and commercialisation, leading to adoption problems downstream

NHS trial approvals process

- **Slow approval processes** (e.g., ethics and data / sample consenting) raise costs of clinical evidence collection (especially for SMEs), delaying adoption
  - Particularly, increased restrictions in **access to tissue / blood** for diagnostics development.

Lack of patent protection (e.g., in-house tests)

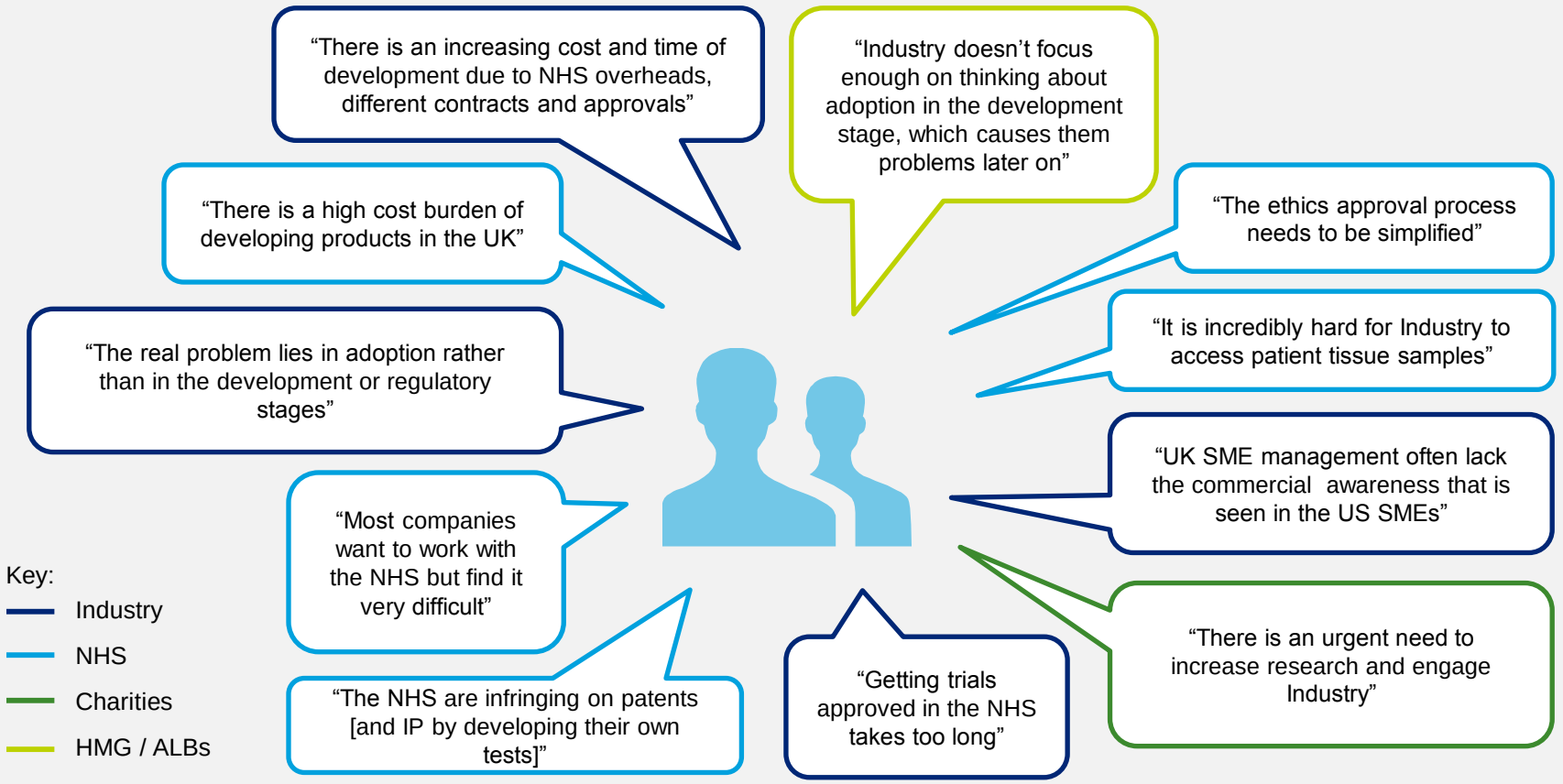
- *In vitro* diagnostic tests can suffer from **lack of strong IP protection** with loss of exclusivity to **in-house testing' by hospital labs**, dis-incentivizing investment in development
  - This problem is **exacerbated by long NICE assessment times**, which can result in **rivals coming to market** before the original test has **completed NICE assessment**

Note: In-house tests are tests developed by the NHS in-house (i.e. within pathology laboratories of individual NHS trusts)
Source: Stakeholder interviews, workshops and Deloitte analysis

Development: *Illustrative Quotes*

Quotes summarise some of the key challenges in the development stage for innovative *in vitro* diagnostics

COMMENTS ON DEVELOPMENT



 Source: Stakeholder interviews, workshops and Deloitte analysis

Stakeholder Views

INDUSTRY VIEWS

- Most Industry stakeholders believe the **NHS trials approvals process is too time consuming**
- The **cost of clinical trials** is a **burden for SMEs**
- Industry feel that there are **limited incentives** for Trusts and healthcare professionals to get **involved with clinical trials**
- **Successful** companies **focus** on **generating evidence of clinical benefit** and are clear about their **route to adoption**
- Industry feel that the **NHS infringes on its IP** by developing some in-house tests, but **Industry rarely takes action** against the NHS out of fear of damaging relationships

HMG / ALBs VIEWS

- HMG / ALBs are of the view that **Industry do not focus** enough time and resources to think about their **route to market** in the development stage, which leads to **later adoption problems**
- HMG / ALBs think that **companies do not** always **see the value** of early **dialogue** conversations with HMG / ALBs, especially around study design and evidence requirements
- **Fees** are a potential **barrier for SMEs** to engage earlier with HMG / ALBs
- Sense that **commercialisation capability** is a problem, especially for **SMEs**

NHS VIEWS

- There is a feeling that it **does not matter how much evidence** manufacturers gather in the development stage as **local commissioners and providers** will want to **re-validate locally**
- It is incredibly **hard for Industry to access patient tissue samples** for research purposes
- Most companies want to work with the NHS but find it **hard to partner with the NHS**
- **Staff capacity limits** the **NHS's ability** to work in the development of innovative *in vitro* diagnostics
- The NHS **infringe** on Industry **patents** with some in-house tests

CHARITY VIEWS

- Charities feel that there is an urgent need to **increase research and engage Industry**, especially for disease areas where there has been no or limited treatment or care advances in the last decade
- The NHS should **encourage more patients and clinicians** to get **involved** with **clinical research**
- **Specific diseases** could be used as a **'test-bed' for reform** from development through to adoption
- The review should **build on** what is **already working**

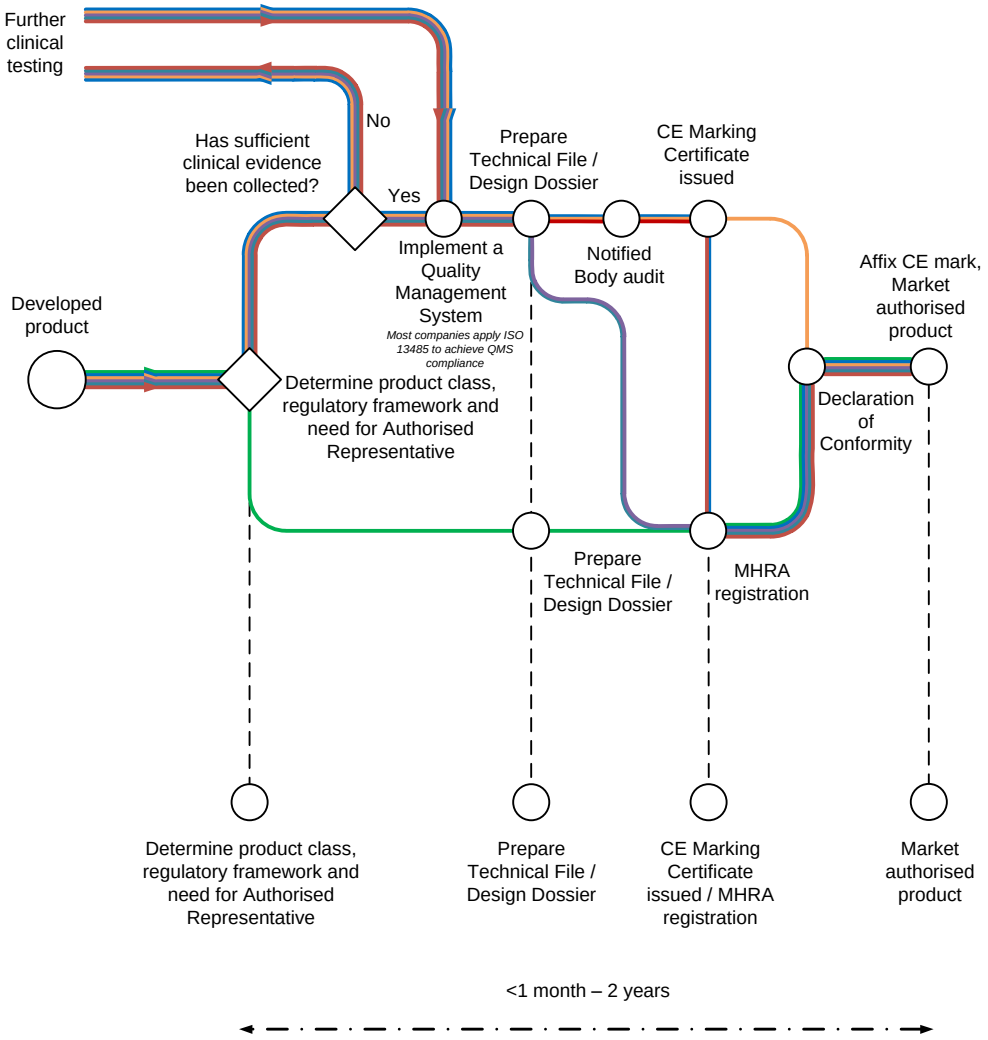
 Source: Stakeholder interviews, workshops and Deloitte analysis

In vitro Diagnostics: Regulation

Regulation: *Detailed Pathway*

Key

- Non-sterile non-measuring Class I devices
- Sterile or measuring Class I devices
- Class II & Class III devices (including radiation-based diagnostics)
- General non-companion *in vitro* diagnostics
- General companion *in vitro* diagnostics
- High risk *in vitro* diagnostics (including self-testing)



77 Source: Stakeholder interviews, workshops and Deloitte analysis

Key Challenges

Our research highlighted two key challenges in the regulatory stage for the early access to innovative *in vitro* diagnostics

Lack of early dialogue

- **Low uptake of available advice** on protocol and trial design
  - **Not seen as value-add** for companies with deep experience
  - Seen as **too large a time and cost investment** for SMEs
  - Lack of engagement and **uncertainty about effectiveness** of this dialogue reduces partnering

Uneven requirements *vs.* in-house tests

- **Commercially developed** *in vitro* diagnostics are required to **generate evidence for CE marking**. This is **not a** requirement for equivalent tests **developed in-house** by healthcare providers, leading to **disincentives for Industry investment**, and potential quality issues

Potential for greater regulatory burden

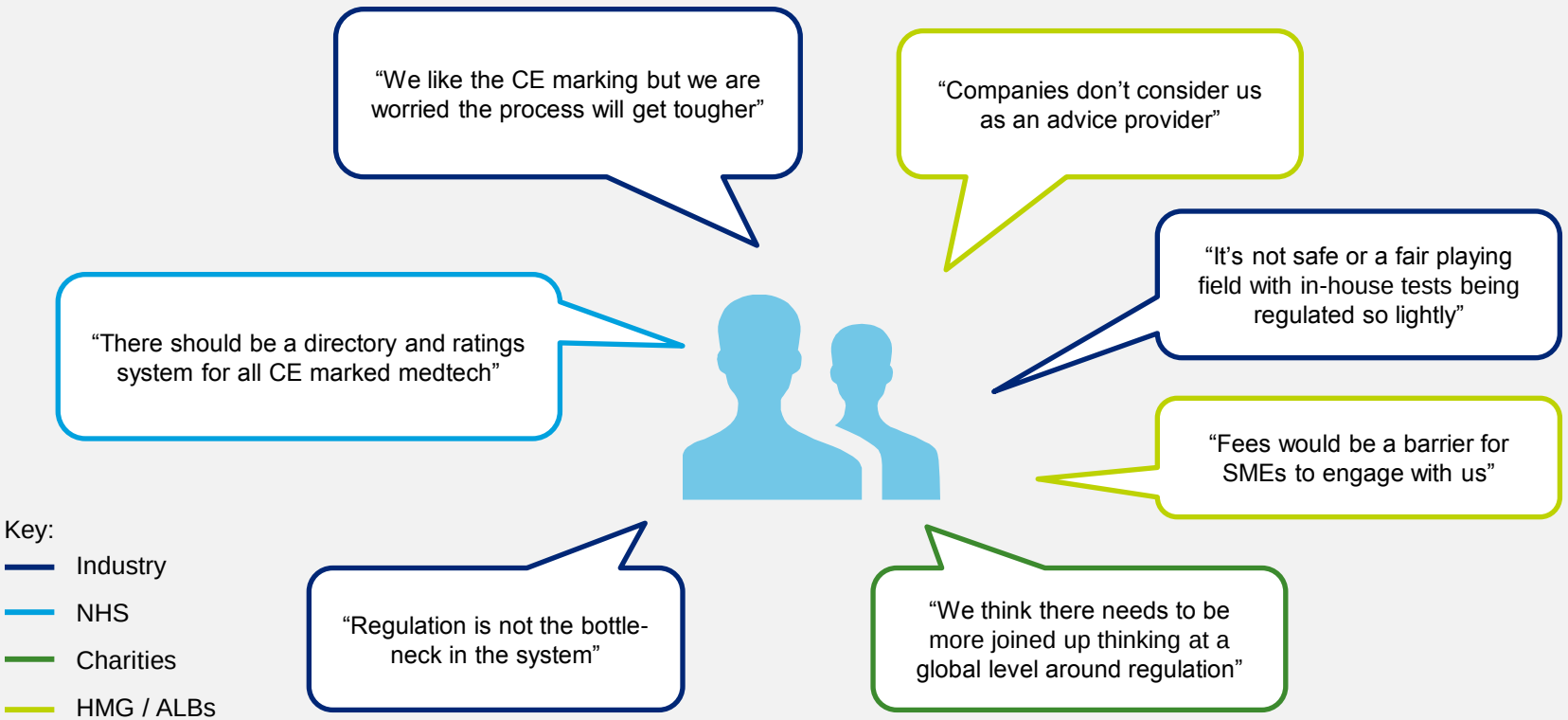
- The **European Union** is currently finalising a **new regulatory Directive** for *in vitro* diagnostics, which is planned for completion in 2016 and **implementation by 2021**
  - Some Industry bodies have raised concerns that this **new system has the potential to significantly increase the evidence requirements** of CE marking without requiring the same of **'in-house' diagnostics**

 Source: Stakeholder interviews, workshops and Deloitte analysis

Regulation: *Illustrative Quotes*

Quotes summarise some of the key challenges in the regulatory stage for innovative *in vitro* diagnostics

COMMENTS ON REGULATION



 Source: Stakeholder interviews, workshops and Deloitte analysis

Stakeholder Views

INDUSTRY VIEWS

- Most Industry stakeholders **do not believe regulation is too onerous**
- However, there is a feeling that the lower **evidence requirements for CE marking** compared to reimbursement and commissioning procedures **result in later adoption problems** where studies have focused on **meeting regulatory requirements** rather than reimbursement and commissioning evidence requirements
- **Limited early dialogue** between Industry and HMG / ALBs
- Feel that NHS **in-house testing** is **not adequately regulated**
- Concerned that **new EU regulation** will **increase costs**

HMG / ALBs VIEWS

- HMG / ALBs are of the view that **Industry do not focus** enough time and resources thinking about their **route to market** in the regulatory stage, which leads to **later adoption problems**
- HMG / ALBs think that **companies do not** always **see the value** of early **dialogue** conversations with HMG / ALBs
- **Fees** are a potential **barrier for SME** early engagement
- Feel that Industry generally just considers the **MHRA** as a **regulatory authority**, rather than an **agency** that is **able to provide advice**

NHS VIEWS

- There is the feeling in the NHS that the **regulatory stage** is the stage that is **least challenging**
- There is currently **not a list of all CE marked *in vitro* diagnostics**, which means adoption often relies on Industry push rather than NHS pull

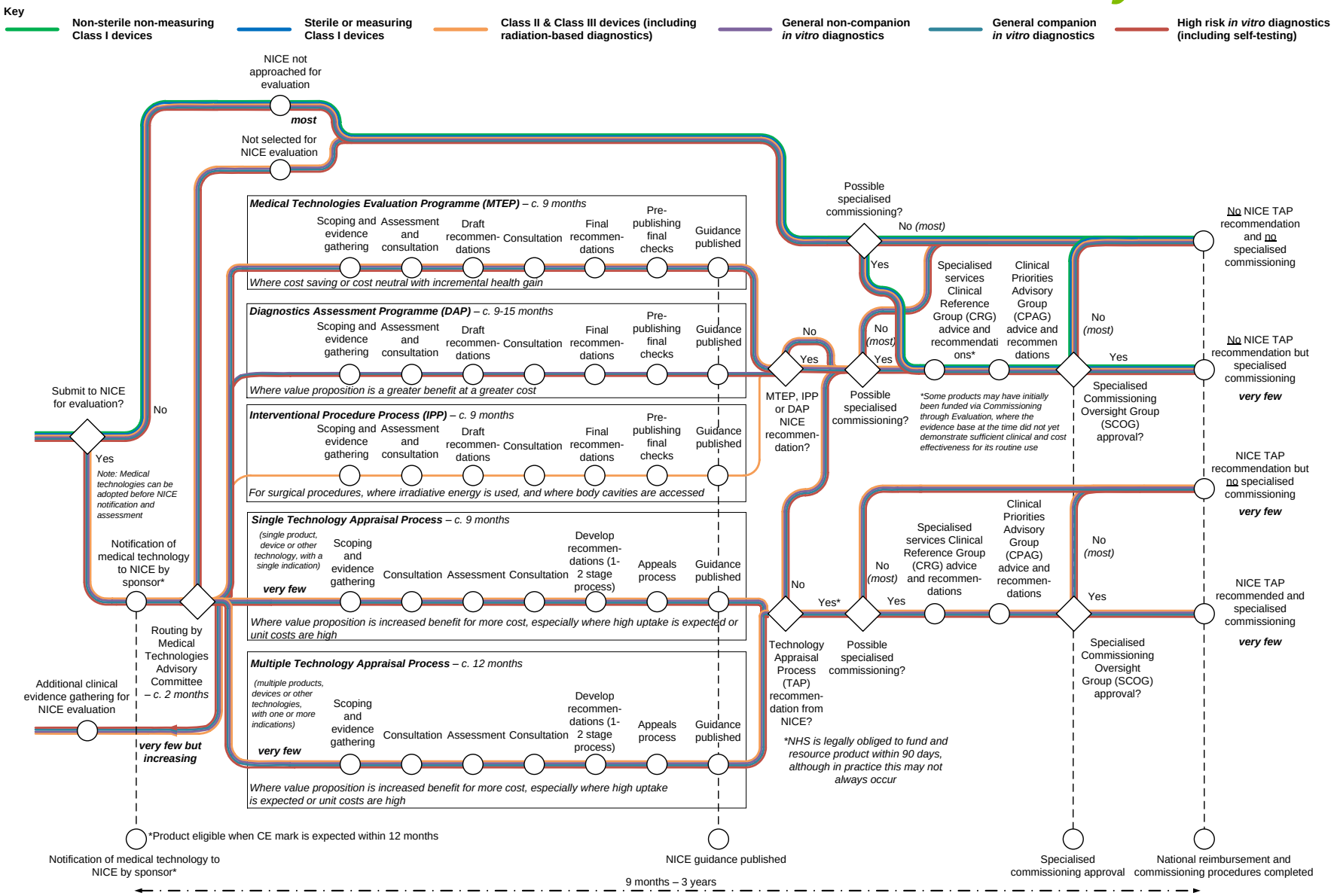
CHARITY VIEWS

- Demand for more **joined up thinking** around **regulation globally**
- **Specific diseases** could be used as a **'test-bed' for reform** from development through to adoption
- The review should **build on** what is **already working**

 Source: Stakeholder interviews, workshops and Deloitte analysis

In vitro Diagnostics: National Reimbursement

National reimbursement: *Detailed Pathway*



 Source: Stakeholder interviews, workshops and Deloitte analysis

Key Challenges

Our research highlighted four key challenges in the national reimbursement and commissioning stage for the early access to innovative *in vitro* diagnostics

Lack of funding mandate

- ***In vitro* diagnostics** recommended by the **DAP do not receive a funding mandate**, with limited effect on local adoption
- Where a **drug and its CDx is recommended** through the NICE TAP, there is **no funding mandate for the CDx** (while the drug component has a funding mandate)
  - In practice, we've heard that TAP recommended CDx are **not being funded locally** or being **funded by pharma**

Lack of commercialisation capability

- Manufacturers, especially SMEs, and healthcare professionals sometimes **struggle to articulate and measure the value proposition of *in vitro* diagnostics**, especially during processes originally designed for pharmaceuticals and when the value realised by a diagnostic may take several years to realise

System requires Industry push

- Suggestion that **Industry** (especially SMEs) are **unwilling to engage with NICE** at early dialogue opportunities or submit products for assessment due to **concerns about success rates** and the **additional cost and time** of gathering sufficient evidence for the NICE assessment process

Usual funding streams don't cover CDx

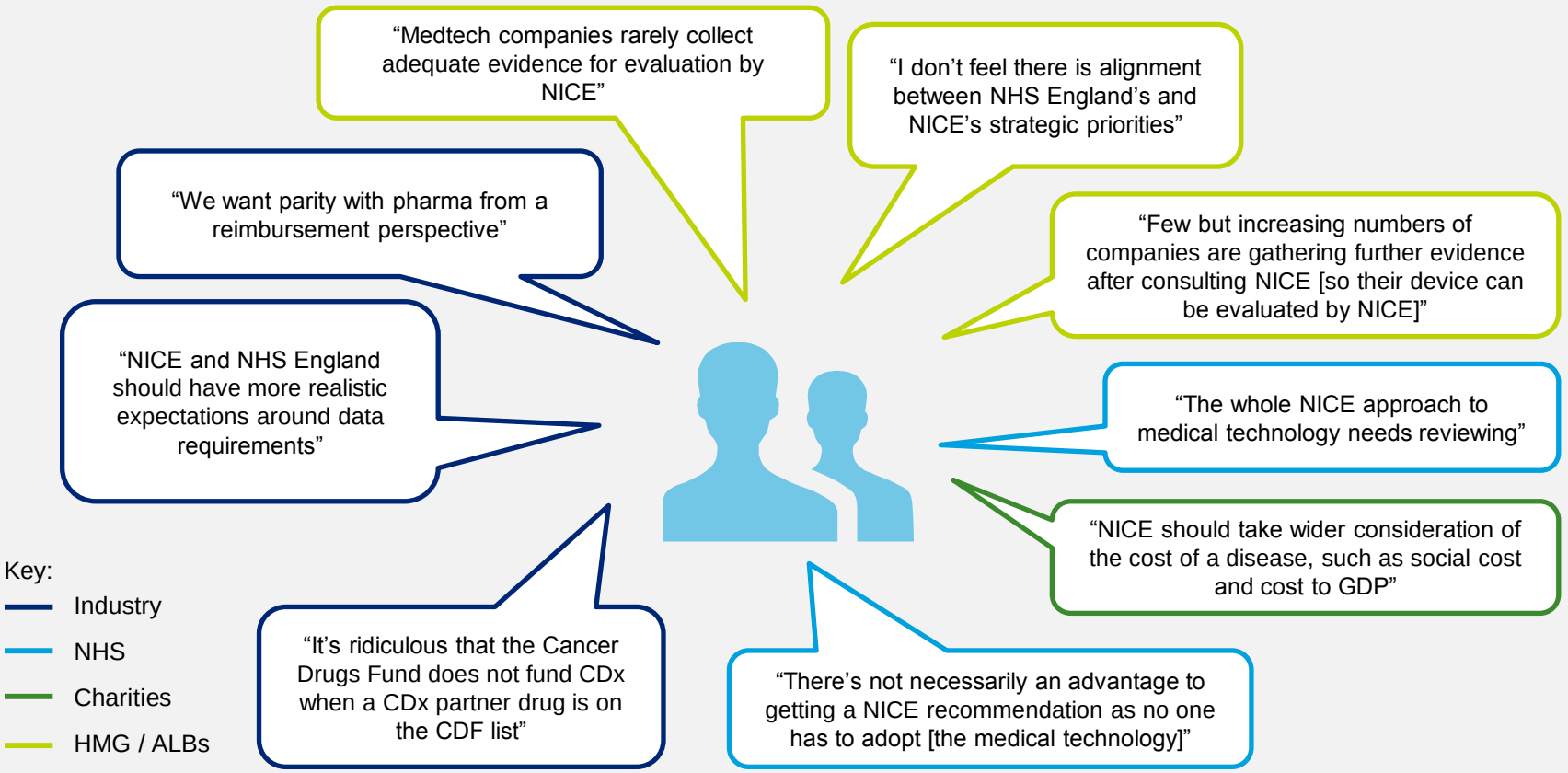
- Currently, **pharmaceutical companies pay for a considerable proportion of companion diagnostic tests** due to uncertainties around the funding of companion diagnostics by the NHS (e.g., the **Cancer Drugs Fund** does not fund CDx even when the partner drug is funded)

Notes: CDx = co-diagnostics; TAP = Technology Appraisal Programme; DAP = Diagnostics Assessment Programme
Source: Stakeholder interviews, workshops and Deloitte analysis

National Reimbursement: *Illustrative Quotes*

Quotes summarise some of the key challenges in the national reimbursement stage for innovative *in vitro* diagnostics

COMMENTS ON NATIONAL REIMBURSEMENT



Source: Stakeholder interviews, workshops and Deloitte analysis

Stakeholder Views

INDUSTRY VIEWS

- Industry **would like NICE-recommended** *in vitro* diagnostics to have a **funding mandate**
- Feeling that *in vitro* diagnostics **value propositions can be hard to articulate** in processes that were originally designed for pharma
- Feeling that **NICE turnaround times can be too long**, especially when compared to the innovation cycle
- Suggestion that Industry (especially SMEs) are **unwilling to engage with NICE** at early dialogue opportunities or submit products for assessment due to **concerns about success rates** and the **additional time and cost** of gathering further evidence

HMG / ALBs VIEWS

- There is the general belief that there **could be better alignment between HMG /ALBs** (e.g., between NHS England, NICE and the Department of Health)
- Suggestion that NICE should be having **early dialogue** with Industry in the regulatory and development stages so that the correct advice and **guidance on evidence collection** for national reimbursement and local adoption purposes can be provided
- Companies should be **encouraged to submit to NICE** at the same time that companies begin the CE marking process

NHS VIEWS

- There is the feeling that in the NHS that there is a **disjoint between national and local organisations**; for instance, a recommendation from NICE does not mean that there will be widespread adoption of an *in vitro* diagnostic across CCGs / providers

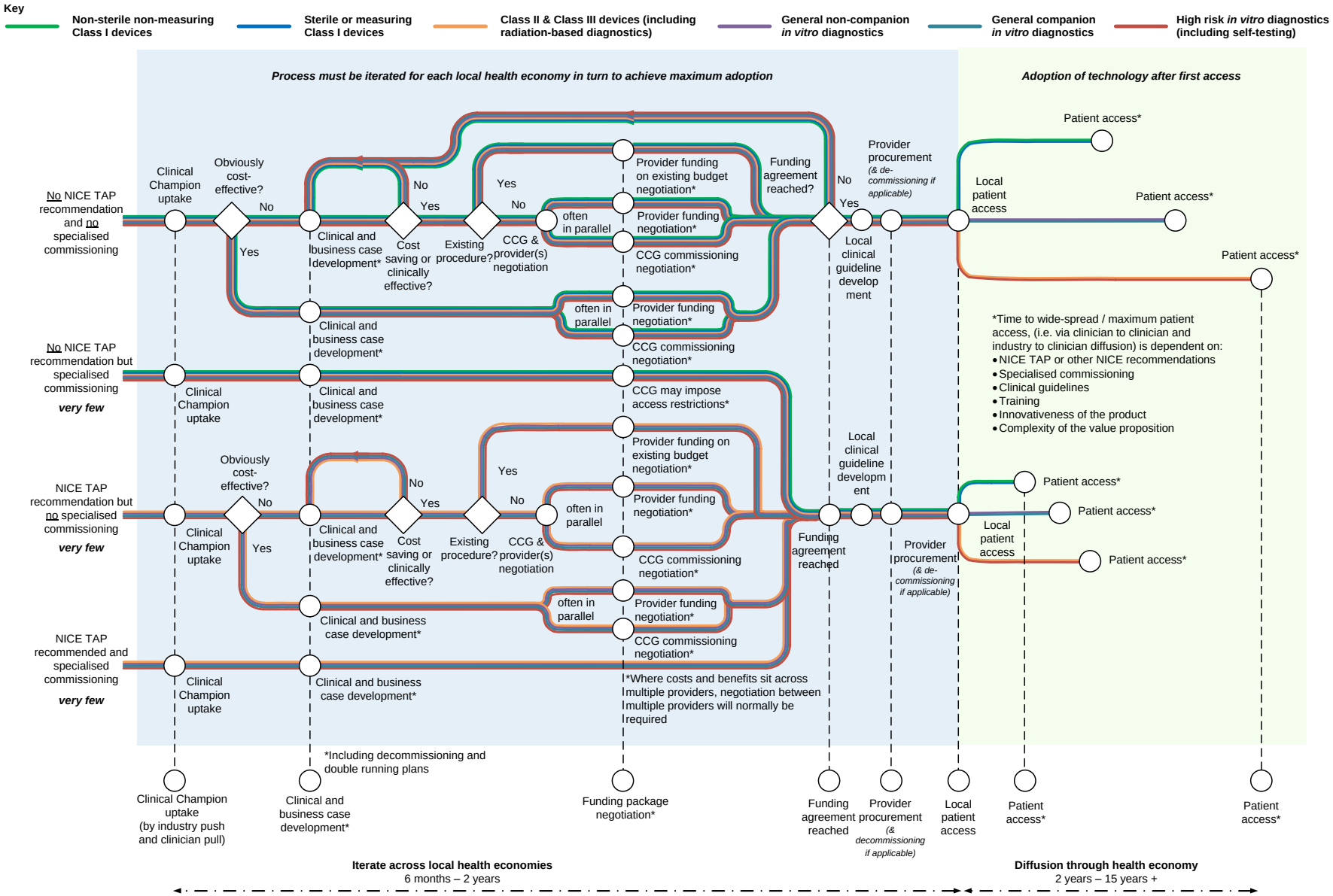
CHARITY VIEWS

- NICE assessment is **not considering** the overall **cost effectiveness** to **patient and society**
- Feeling that NICE **topic selection** does **not focus enough** attention on *in vitro* diagnostics that would only affect a **small group** of patients
- **Specific diseases** could be used as a **'test-bed' for reform** from development through to adoption
- The review should **build on** what is **already working**

 Source: Stakeholder interviews, workshops and Deloitte analysis

In vitro Diagnostics: Local Commissioning & Adoption

Local commissioning & adoption: *Detailed Pathway*



 Source: Stakeholder interviews, workshops and Deloitte analysis

Key Challenges

Our research highlighted four key challenges in the local commissioning and adoption stage for the early access to innovative *in vitro* diagnostics

Unclear route to market

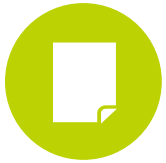
- **No consistent route to local markets** exists for many *in vitro* diagnostics, with a **wide range of contracting arrangements** from simple one-off local orders through to standing orders of tens of millions of pounds. There is also a **heavy reliance of personal relationships** to drive adoption

Process duplication

- **Lack of central coordination** and clear route to market results in significant **sales process duplication** across CCGs and / or providers. Additionally, the process of getting an *in vitro* diagnostic **compatible with many multiplex assay platforms** is costly, especially for SMEs

Budget siloes

- **Internal budget siloes** within and between NHS organisations dis-incentivises the adoption of *in vitro* diagnostics where the **costs and value realised are not aligned** (e.g. pathology *vs* therapeutic / pathway budgets)
  - **Budget short-sightedness** further dis-incentivise investments that would **save costs at an aggregate level**



Contracting

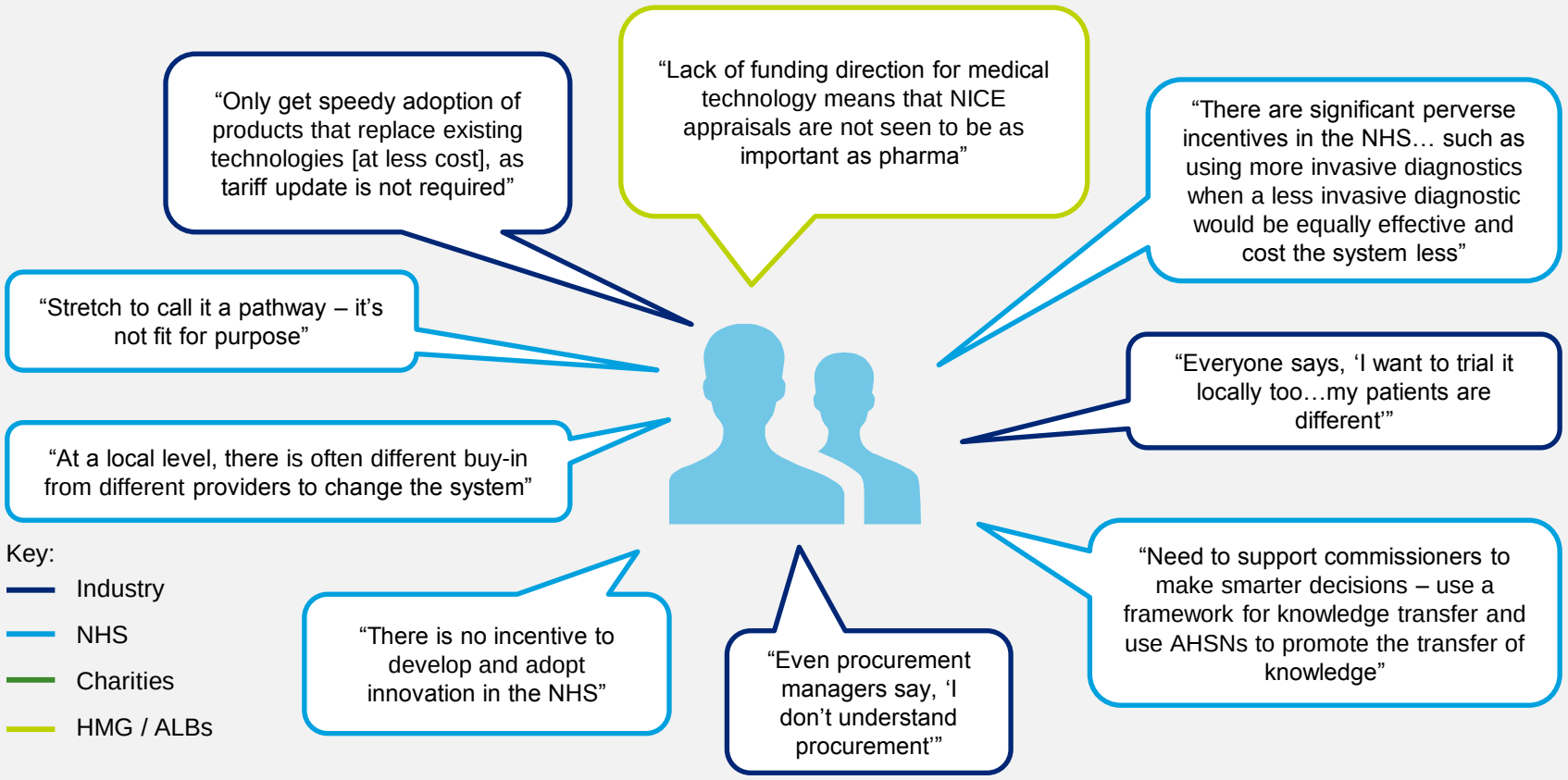
- There is currently **little standardisation of process and documentation for contracts,** which results in the **use of procurement intermediaries** and reduces prices. Additionally, the **use of managed service contracts** with long renewal terms limits innovation, competition and creates **barriers to change** in the NHS (see case study)

 Source: Stakeholder interviews, workshops and Deloitte analysis

Illustrative Quotes

Quotes summarise some of the key challenges in the local commissioning and adoption stage for innovative *in vitro* diagnostics

COMMENTS ON LOCAL COMMISSIONING AND ADOPTION



Source: Stakeholder interviews, workshops and Deloitte analysis

Stakeholder Views

INDUSTRY VIEWS

- Industry stakeholders interviewed are **strongly of the opinion** that local commissioning and adoption of medtech is the **greatest barrier to patient access**
- **No clear route to market**, with high variability between LHEs and **access highly dependent on clinician relationships**
- **Lack of funding mandate** for non-TAP NICE-assessed products limits adoption
- Feeling that *in vitro* diagnostics are just **considered a system overhead cost by commissioners**
- **Little contract standardisation**, which particularly affects SMEs

HMG / ALBs VIEWS

- Agreement among interviewed stakeholders that **adoption**, especially at local level is **complex and slow**
- **Lack of a clear route to market** identified as a problem
- Acceptance that **lack of funding mandate** for most NICE-recommended *in vitro* diagnostic limits local commissioner willingness to pay for innovative *in vitro* diagnostics
- Feeling that **lack of central coordination** results in significant **duplication** across CCGs and / or providers
- Sense that **commercialisation capability** is a problem, especially for **SMEs**

NHS VIEWS

- **Majority** of interviewed NHS stakeholders believed that local commissioning and adoption is the **greatest barrier to access**
- **Major barriers** to clinician use of *in vitro* diagnostics are **financial**:
  - **Speed of pricing** and **contracting** update (e.g., inclusion on Tariff) even for NICE-recommended products
  - **Departmental and LHE budget silos** and **horizons** limit the uptake of products, especially where benefits will accrue across multiple care pathways and settings of care
  - **Double running costs** when changes occur

CHARITY VIEWS

- Feeling that NHS commissioners **do not focus enough on patients** and their quality of life, and spend too much time meeting minimum performance standards and financing constraints
- Feel that some **inequalities between LHEs** are due to **variation** in the **adoption** of innovative medtech
- **Specific diseases** could be used as a **'test-bed' for reform** from development through to adoption

Notes: LHE = Local Health Economy; TAP = Technology Appraisal Programme
Source: Stakeholder interviews, workshops and Deloitte analysis

In vitro Diagnostics: Case studies

Case study – *In vitro* diagnostics (1/2)

A non-invasive pre-natal test has had limited adoption due to discussions around the inclusion in the national Tariff for the maternity pathway

Background

- Non-Invasive Pre-Natal Testing (NIPT) uses cell-free foetal DNA from a mother's blood sample to analyse chromosomal abnormalities such as Down's Syndrome, Edwards Syndrome and Patau Syndrome. The test is less invasive than the alternative amniocentesis/CVS, currently offered for mothers with above 1:150 probability
- An NHS hospital has developed the capabilities to deliver the test in their lab
- The test is not offered as part of the current NHS maternity pathway and the RAPID trial has been designed to gather evidence
- The inclusion in the NHS maternity pathway and overall widespread adoption is the main challenge

Issues

- The new service at the offered price is less than the invasive test for the NHS, but adoption at Trust level has not been widespread
- As a new service development, Trusts need to discuss the introduction of NIPT with CCGs to be included as part of the maternity pathway and to be paid under the national tariff
- Trusts and CCGs are not considering whole system costs as they only look at the maternity pathway and not all the additional costs to the system
- The inclusion in the National Screening Programme is a lengthy process which leads to adoption in some areas of England, increasing the inequality access to services

Challenges

Counterproductive incentives

Lack of funding mandate

Usual funding streams don't cover CDx

Unclear route to market

Process duplication

Budget siloes

Contracting

92 Source: Stakeholder interviews, workshops and Deloitte analysis

Case study – *In vitro* diagnostics (2/2)

The use of a calprotectin test on individuals with chronic intestinal symptoms saves money and is recommended by NICE, but tests are not widely used

Background

- In primary care a common diagnostic challenge is the management of patients with chronic intestinal symptoms, in particular the differentiation of IBD, which includes both Crohn's Disease (CD) and Ulcerative Colitis (UC), from IBS
- There are simple, accurate and differentiating tests, to aid in management and referral decisions for such patients (calprotectin tests) that are not widely used
- Referral to secondary care is straightforward in patients with acute symptoms or "red flag" features, such as blood in stool, nocturnal symptoms, weight loss, family history of colon cancer or presence of clinical signs
- However, the majority of patients comprise a mixed group with both identifiable pathologies and "functional" condition

Issues

- The use of a calprotectin test on individuals with chronic intestinal symptoms saves money downstream in terms of an outpatient appointment, endoscopy and follow up appointments
- Cost benefit analysis suggests that widespread adoption of point-of-care calprotectin tests would save the NHS c.£200m but adoption is not widespread due to perverse incentives
- Overall savings for the local health economy are seen as a loss of revenue to Trust budgets, who are paid per episode using the Payments by Results Tariff
- NICE produced guidance in October 2013 recommending the use of faecal calprotectin testing to help doctors distinguish between inflammatory bowel diseases
- Despite the aggregate cost savings and NICE guidance, there has been limited adoption of calprotectin tests

Challenges

Counterproductive incentives

Lack of funding mandate

Usual funding streams don't cover CDx

Unclear route to market

Process duplication

Budget siloes

Contracting

93 Source: Stakeholder interviews, workshops and Deloitte analysis

Contents

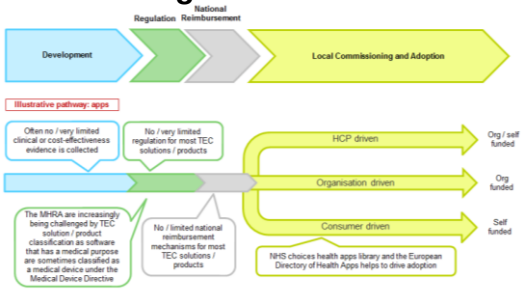
Future proofing

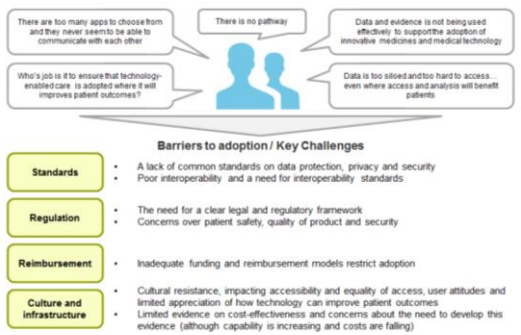
We've considered whether the current pathways for digital health and precision medicine are fit for purpose

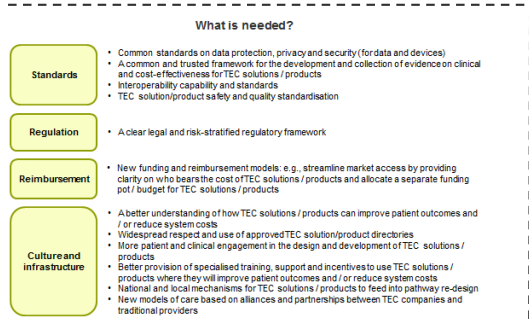
We've considered:

1. The current pathway
2. Key stakeholder views and the current barriers to adoption
3. What is needed to make the pathways future proof

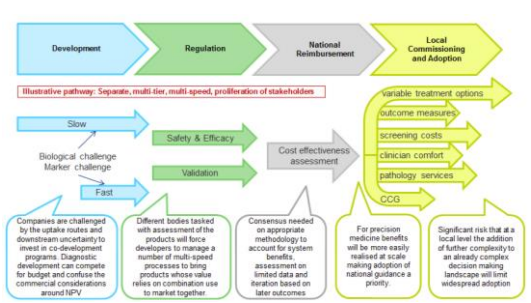
Digital Health

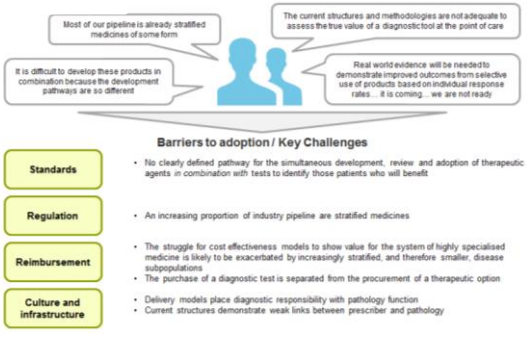


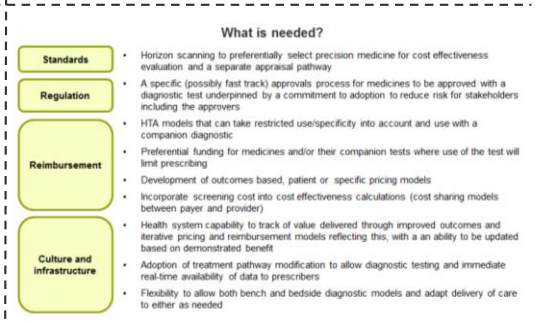




Precision Medicine



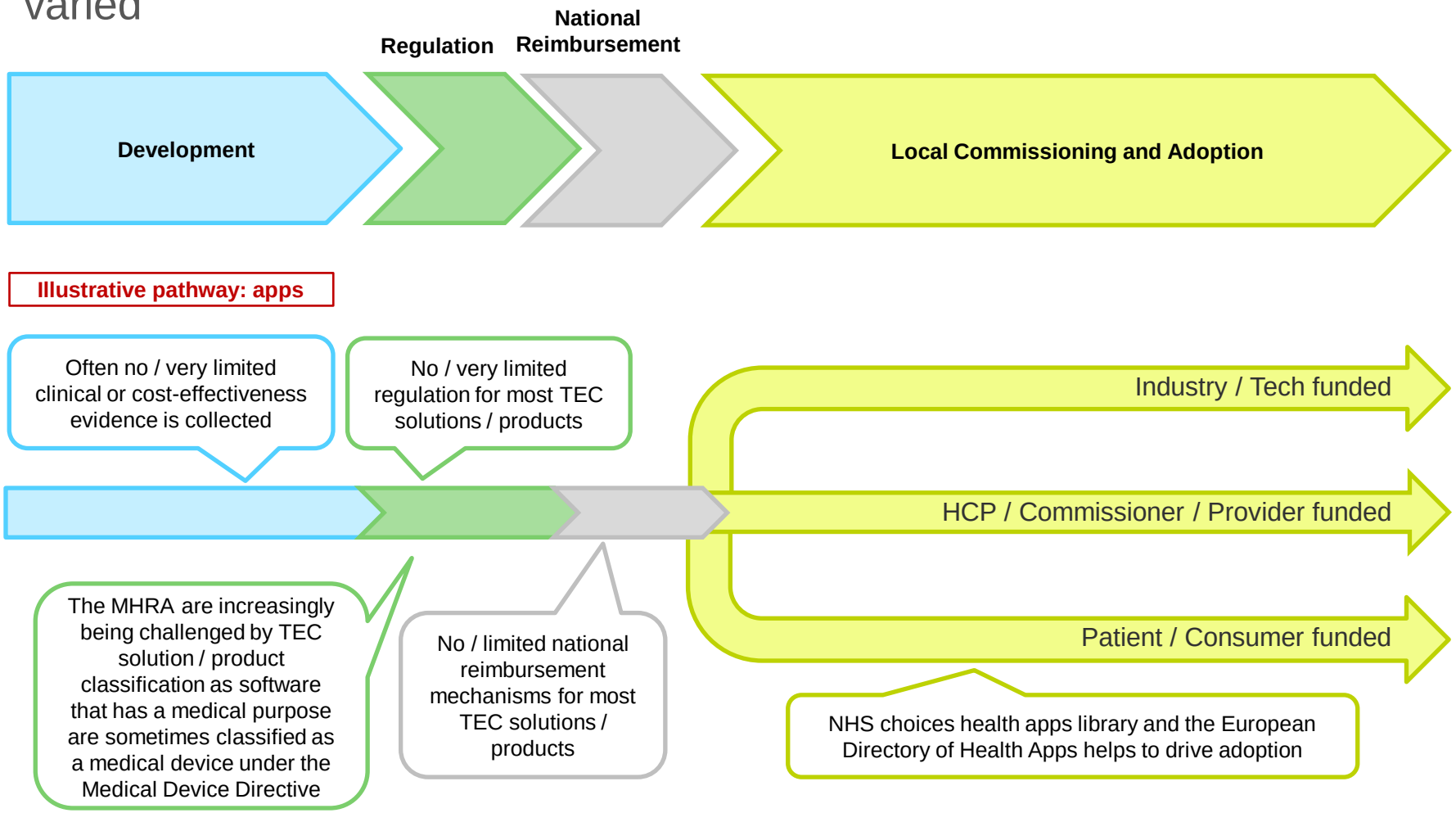




 Source: Stakeholder interviews, workshops and Deloitte analysis

Future proofing: *Digital health*

The current pathway from development to adoption for digital health is varied



Notes: TEC = Technology-Enabled Care; Currently some consumer wearables and apps will fall within the regulatory definition of a medical device; Stakeholder interviews, workshops and Deloitte analysis

Future proofing: *Digital health*

The digital health pathway is not fit for purpose with a number of significant barriers to widespread adoption

Barriers to adoption / Key Challenges

Standards	• A lack of common standards on data protection, privacy and security • Poor interoperability and a need for interoperability standards
Regulation	• The need for a clear legal and regulatory framework • Concerns over patient safety, quality of product and security
Reimbursement	• Inadequate funding and reimbursement models restrict adoption
Culture and infrastructure	• Cultural resistance, impacting accessibility and equality of access, user attitudes and limited appreciation of how technology can improve patient outcomes • Limited evidence on cost-effectiveness and concerns about the need to develop this evidence (although capability is increasing and costs are falling)

Note: TEC = Technology-Enabled Care
Source: Stakeholder interviews, workshops and Deloitte analysis

Future proofing: *Digital health*

There are a number of barriers that need to addressed for the digital health pathway to be future proofed

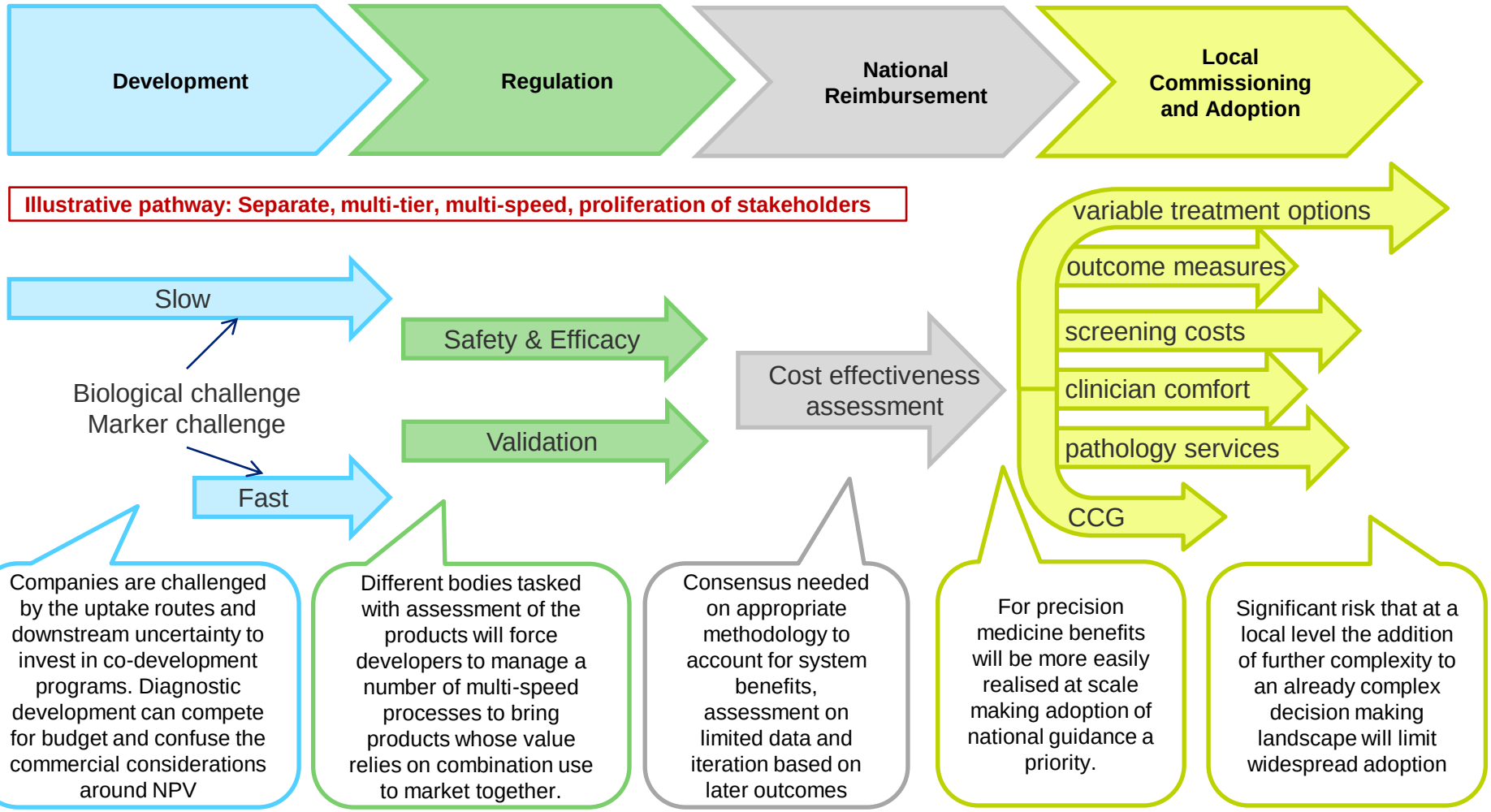
What is needed?

Standards	• Common standards on data protection, privacy and security (for data and devices) • A common and trusted framework for the development and collection of evidence on clinical and cost-effectiveness for TEC solutions / products • Interoperability capability and standards • TEC solution/product safety and quality standardisation
Regulation	• A clear legal and risk-stratified regulatory framework
Reimbursement	• New funding and reimbursement models: e.g., streamline market access by providing clarity on who bears the cost of TEC solutions / products and allocate a separate funding pot / budget for TEC solutions / products
Culture and infrastructure	• A better understanding of how TEC solutions / products can improve patient outcomes and / or reduce system costs • Widespread respect and use of approved TEC solution/product directories • More patient and clinical engagement in the design and development of TEC solutions / products • Better provision of specialised training, support and incentives to use TEC solutions / products where they will improve patient outcomes and / or reduce system costs • National and local mechanisms for TEC solutions / products to feed into pathway re-design • New models of care based on alliances and partnerships between TEC companies and traditional providers

Note: TEC = Technology-Enabled Care
Source: Stakeholder interviews, workshops and Deloitte analysis

Future proofing: *Precision Medicine/Companion Diagnostics*

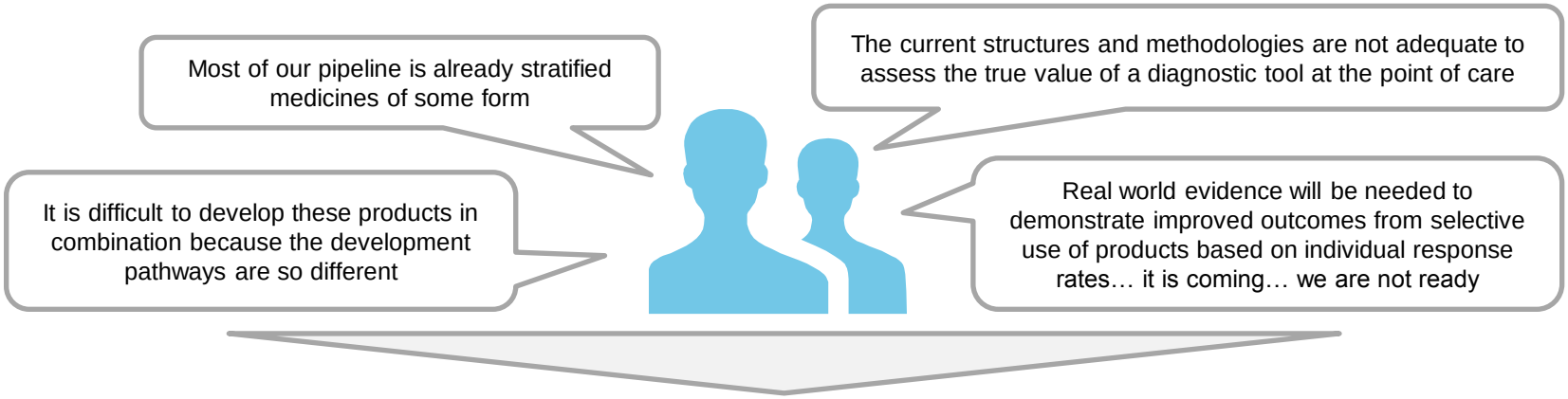
Precision medicine requires the system to reward innovations that by definition reduce the number of recipients of a given treatment option



 Source: Stakeholder interviews, workshops and Deloitte analysis

Future proofing: *Precision medicine and companion diagnostics*

A rapidly developing technical ability to stratify patients suitability of treatment options will require paradigm shift in development and adoption



Barriers to adoption / Key Challenges

Standards

- No clearly defined pathway for the simultaneous development, review and adoption of therapeutic agents *in combination with* tests to identify those patients who will benefit

Regulation

- An increasing proportion of Industry pipeline are stratified medicines

Reimbursement

- The struggle for cost effectiveness models to show value for the system of highly specialised medicine is likely to be exacerbated by increasingly stratified, and therefore smaller, disease subpopulations
- The purchase of a diagnostic test is separated from the procurement of a therapeutic option

Culture and infrastructure

- Delivery models place diagnostic responsibility with pathology function
- Current structures demonstrate weak links between prescriber and pathology

 Source: Stakeholder interviews, workshops and Deloitte analysis

Future proofing: *Precision medicine and companion diagnostics*

The promise of precision medicine includes savings through a improved medicines use and a longer timeframe to recoup savings

What is needed?

Standards	• Horizon scanning to identify precision medicines and the implications on the appraisal pathway
Regulation	• A specific (possibly fast track) approvals process for medicines to be approved with a diagnostic test underpinned by a commitment to adoption to reduce risk for stakeholders including the approvers
Reimbursement	• HTA models that can take restricted use/specificity into account and use with a companion diagnostic • Preferential funding for medicines and/or their companion tests where use of the test will limit prescribing • Development of outcomes based, patient or specific pricing models • Incorporate screening cost into cost effectiveness calculations (cost sharing models between payer and provider)
Culture and infrastructure	• Health system capability to track of value delivered through improved outcomes and iterative pricing and reimbursement models reflecting this, with a an ability to be updated based on demonstrated benefit • Adoption of treatment pathway modification to allow diagnostic testing and immediate real-time availability of data to prescribers • Flexibility to allow both bench and bedside diagnostic models and adapt delivery of care to either as needed

 Source: Stakeholder interviews, workshops and Deloitte analysis

Contents

Methodology

To develop the final report the outputs of 4 phases of work were combined for the key challenge themes, case studies and opportunities

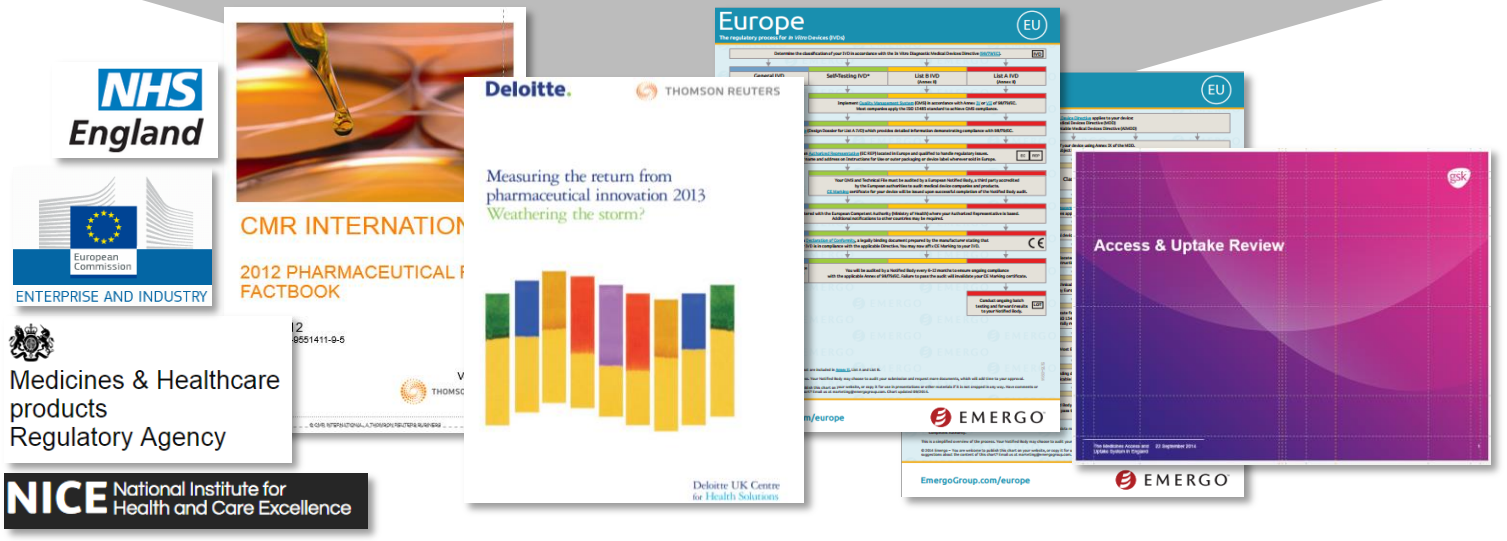
	Desk research	*Interviews*	*Workshops*	*Synthesis*
Why?	• To gather publicly available data • Understand how the current situation should work in paper	• To gather views and opinions of each stakeholder • To compare to the "in paper" current situation	• To test hypothesis, discuss ideas and define priorities • To prioritise opportunities for the next phase	• To identify, refine and prioritise barriers within the UK's current approach • To prioritise opportunities
What?	• Case study research • On-line/web-site navigation • Papers/literature review	• Interviews with main stakeholders involved in the development pathways of medicines, devices and diagnostics	• Interactive sessions, with plenary, break-out sessions and presentations	• Quantitative and qualitative analysis of the key themes from the interviews and workshops
Who?	Led by the Deloitte team insights were gathered from: • Desk research • Materials shared by OLS • Deloitte • CASMI • The King's Fund • Case studies shared by NICE and Industry	• Over 50 people were interviewed from Industry, Public Sector, Academia and Charities by telephone or face-to-face for 30-90 minutes	• 20 participants from Industry, Public Sector, Academia and Charities for a morning session on 20th March • 100 participants from Industry, Public Sector, Academia and Charities for a lunchtime session on 24th March • 15 representatives from AHSNs	• Led by the Deloitte team supported by CASMI and King's Fund

Product timing estimation

Product pathway timing estimation was generated from desk research combined with stakeholder interviews and workshops

Development → **Regulation** → **National Reimbursement** → **Local Commissioning and Adoption**

Product pathway timing was estimated from desk research and triangulated with stakeholder interviews, workshops and Deloitte analysis



Medtech product classification

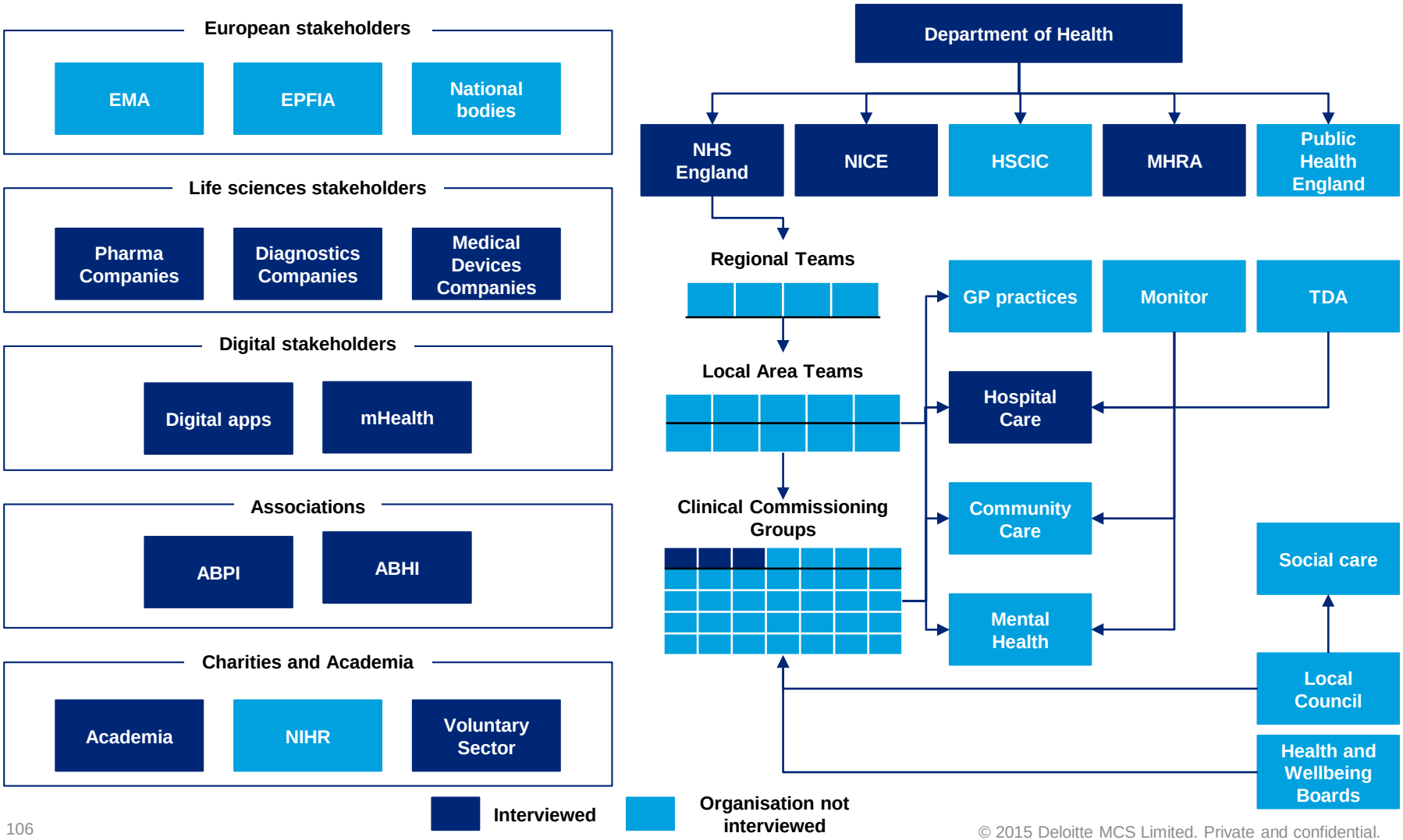
Medtech products have been classified into 6 groups based on the current EU regulatory classification system with the additional of CDx

Medtech product classification		Description	Examples	Key
	Non-sterile non-measuring Class I devices	- Non-invasive devices that either do not touch patients or are only in contact with unbroken skin	• Hospital beds • Wheelchairs	
	Sterile or measuring Class I devices	- Non-invasive measuring devices that touch patients directly or are in contact with broken skin - Invasive devices that are only used transiently in human body cavities	• Scalpels • Forceps • Thermometer	
	Class II & Class III devices (includes radiation-based diagnostics)	- Surgically invasive devices - Implantable or other invasive devices - Active devices that are intended to administer substances, image the human body, or are for direct diagnosis or monitoring purposes	• Muscle stimulators • Hearing aids • Drug eluting stents	
	General non-companion diagnostics *in vitro* diagnostics	- Non-CDx test that samples tissue or body fluid outside of the body	• Blood type tests • Immunity tests	
	General companion diagnostics *in vitro* diagnostics	- in vitro diagnostic that provides info. that is essential for the safe & effective use of a corresponding therapeutic product	• HER2 mutation tests	
	High risk *in vitro* diagnostics (including self-testing)	- Self-testing or in Annex II on List A or B of In-Vitro Diagnostic Devices Directive (98/79/EC)	• HIV tests • Blood glucose tests	

Note: Currently some consumer wearables and apps will fall within the regulatory definition of a medical device
Source: Emergo Group, European Commission, stakeholder interviews, workshops and Deloitte analysis

Innovation stakeholder map

We have interviewed across the UK health economy to assess views on innovation



Glossary

Term	Definition
Adaptive licensing or Agile pathways	Adaptive licensing is a prospectively planned, flexible approach to regulation of drugs and biologics. Through iterative phases of evidence gathering to reduce uncertainties followed by regulatory evaluation and license adaptation, AL seeks to maximize the positive impact of new drugs on public health by balancing timely access for patients with the need to assess and to provide adequate evolving information on benefits and harms so that better-informed patient-care decisions can be made
Biologics	Biologics are a class of large molecule drugs based on proteins that have a therapeutic effect. They are typically copies or optimised versions of endogenous human proteins (e.g., insulin, trastuzumab)
Digital Health	Digital healthcare involves the use of information and communication technologies to help address the health problems and challenges faced by patients. These technologies include both hardware and software solutions and services (e.g., wearables, apps)
Early dialogue	An early dialogue allows input from HTA bodies on the development of the health technology. Early dialogue is prospective in nature as it focuses on development strategies and not on pre-evaluation of data
Genomics	Genomics is defined as the study of genes and their functions, and related techniques. The main difference between genomics and genetics is that genetics scrutinizes the functioning and composition of the single gene where as genomics addresses all genes and their inter relationships in order to identify their combined influence on the growth and development of the organism
In vitro diagnostics	*In vitro* diagnostics refers to a wide range of medical laboratory tests that are used to diagnose diseases and monitor the clinical status of patients using samples of blood, cells or other tissues obtained from a patient
In-house tests	In-house tests are tests developed by the NHS in-house (i.e. within pathology laboratories of individual NHS trusts)
Precision (previously stratified) medicine	Precision medicine is the use of genomic, epigenomic, exposure, and other molecular information to select the best therapeutic strategy in order to improve health outcomes, such as effectiveness and safety, for a targeted group of patients sharing similar biological characteristics
Medical devices	A medical device is an instrument, apparatus, implant, in vitro reagent, or similar or related article that is used to diagnose, prevent, or treat disease or other conditions, and does not achieve its purposes through chemical action within or on the body (which would make it a drug). For the purposes of this report, medical devices do not include *in vitro* diagnostics
Real world evidence (or data)	Real world evidence or data is evidence or data that are collected outside the controlled constraints of conventional randomised controlled trials to evaluate what is happening in normal clinical practice
Small molecules	A small molecule is a low molecular weight compound that regulates a biological process through binding to the target protein in the human body. They are typically chemically manufactured active substance (e.g., aspirin, paracetamol)
Vaccines	Vaccines are biological preparations that provide active acquired immunity to a particular disease. They typically contain an agent that resembles a disease-causing microorganism (e.g., flu vaccine, Hepatitis B vaccine)

Limitations to the pathway analysis

Limitations of the pathways shown in this document.

1. The pathways included in this document are generalised and are created using desk research, stakeholder involvement and Deloitte analysis.

2. They are illustrative and can not be considered exhaustive.

3. To simplify the pathway some non-linear elements of the pathway are shown in a linear manners

Monitor Deloitte.