Medicines Horizon Scanning Annual Report 2021

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Background

The Innovation Observatory (IO) is a national horizon scanning research centre funded by the National Institute for Health and Care Research (NIHR) hosted at Newcastle University. The IO focuses on identifying emerging health innovations that are in the pipeline and providing early awareness signals and access to timely intelligence to stakeholders including the National Institute for Health and Care Excellence (NICE) and the Innovation, Research and Life Sciences and Strategy (IRLSS) group to support decision making around healthcare innovation. The IO also proactively advances cutting edge horizon scanning research methods and tools that allow more effective and efficient identification of emerging health innovations, enable efficiency in the innovation, regulation and market access pathway along with current and future trends thus playing a vital role in supporting getting the right innovation to patients at the right time.

Within the IO, one of the areas of research is the Medicines Programmes that aims to identify innovative medicines that meet the needs of the NHS and are likely to be available for market access in the UK or EU in the next few years. Innovative medicines comprise new medicinal products that are not yet licensed for any indication along with already licensed medicinal products that are now in clinical development for new indications.

Each year, the Medicines Horizon Scanning (MHS) team identifies and sifts through nearly 5,000 clinical trials to identify those in remit for us to monitor and track through well-established horizon scanning processes. Each year, the Medicines Programme provides technology briefings on up to 200 medicinal innovations to the National Institute for Health and Care Excellence (NICE) and other scans to requested by stakeholders such the NHS and IRLSS group.

Aim

This report provides an overview of the MHS team's horizon scanning activity in 2021. It provides: a summary of the horizon scanning strategy devised to identify newly created or updated[1] records within 2021; an overview of the landscape of clinical trials in remit, the therapeutic areas of the indications based on NICE condition categories,[1,2] drug designations, industry developers; and gives some high-level conclusions drawing on the themes and patterns identified.

Methods

1. Medicines Innovation Database

The IO has developed a bespoke, comprehensive database, the Medicines Innovation Database (MInD) that serves as a repository of intelligence collected from:

- Hard data sources (clinical trial registries, regulatory authorities)
- Soft intel (news/media)
- Pharma intel (company website, press releases, direct engagement with companies)

[1] An update constitutes addition of a newly identified clinical trial relevant to a record present on MInD

The MInD consists of unique records [innovative medicine(s) + target indication] that provide a clear and complete picture of the clinical/innovation profile and regulatory status of each medicine in clinical development. The information collated on MInD helps provide the most reliable and accurate intelligence for medicines in development for national stakeholders to inform timely assessment and facilitate decision making.

2. Scope:

Medicinal products in clinical development that fall within the scope of the MHS team at the IO need to fulfil the following criteria:

- Medicinal products and therapeutic vaccines (vaccine used during disease state)
- Interventional trials
- Phase I/II to III trials
- Trial locations in Europe, US, Canada

Therapeutic and prophylactic interventions for COVID-19 identified from March 2020 to March 2023 were regularly collated in a separate scan that was sent to the RAPID C-19[2] team on a weekly (later monthly) basis, and also uploaded on the IO website as the COVID-19 dashboard.[3]

3. Search Strategy for this report:

The MInD was queried to identify newly created records and updated records from 1st January – 31st December 2021. Most of the new records were created from clinical trials sifts. A small number were created from new records identified from UK PharmaScan, news articles or information provided by pharmaceutical companies during pipeline meetings.

Analysis of 2021 landscape of medicines in remit for IO

Our analysis showed that from a total of 1,925 records, 1,122 were created and 803 were updated in 2021 on MInD.

We consider these new and updated records in relation to the indications for which they are in development; the types of technologies under development; the regulatory status of the technologies and specific populations involved.

1. Indications

i. Cancer vs non-cancer indications

The 2021 records included 824 (43%) and 1,101 (57%) cancer and non-cancer records respectively (figure 1). Figure 2 shows the cancer and non-cancer indications as classified by NICE categories. The cancer indications with the most records included blood and bone marrow cancers, multiple cancers, and lung cancers. Within blood and bone marrow cancers,

[2] RAPID C-19 was a group set early in the pandemic to get treatments for COVID-19 to patients quickly and safely and included NHS England, Medicines and Healthcare products Regulatory Agency, National Institute for Health and Care Research, Scottish Medicines Consortium (Healthcare Improvement Scotland), All Wales Therapeutics and Toxicology Centre, All Wales Medicines Strategy Group, Department of Health in Northern Ireland, Antivirals and Therapeutics Task Force at the Department of Health and Social Care

the indications with the most number of records were multiple myeloma, acute myeloid leukaemia, and diffuse large B-cell lymphoma. Non-cancer indications with the highest number of records included, neurological conditions, diabetes and other endocrinal, nutritional and metabolic conditions. Within neurological conditions, the indications that were the most common were Parkinson's disease, multiple sclerosis, and Alzheimer's disease.

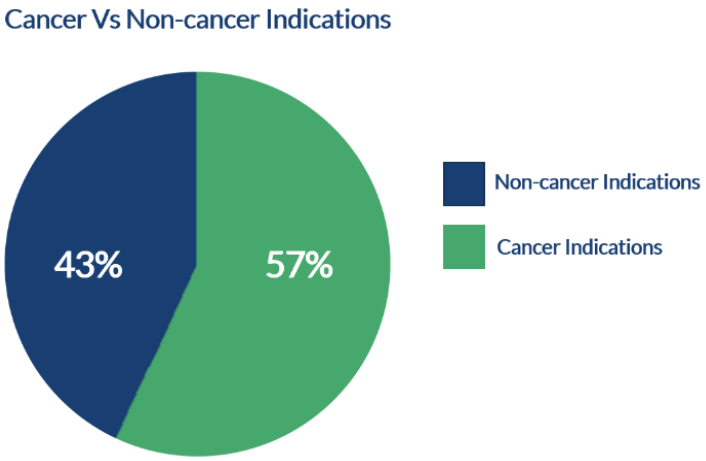
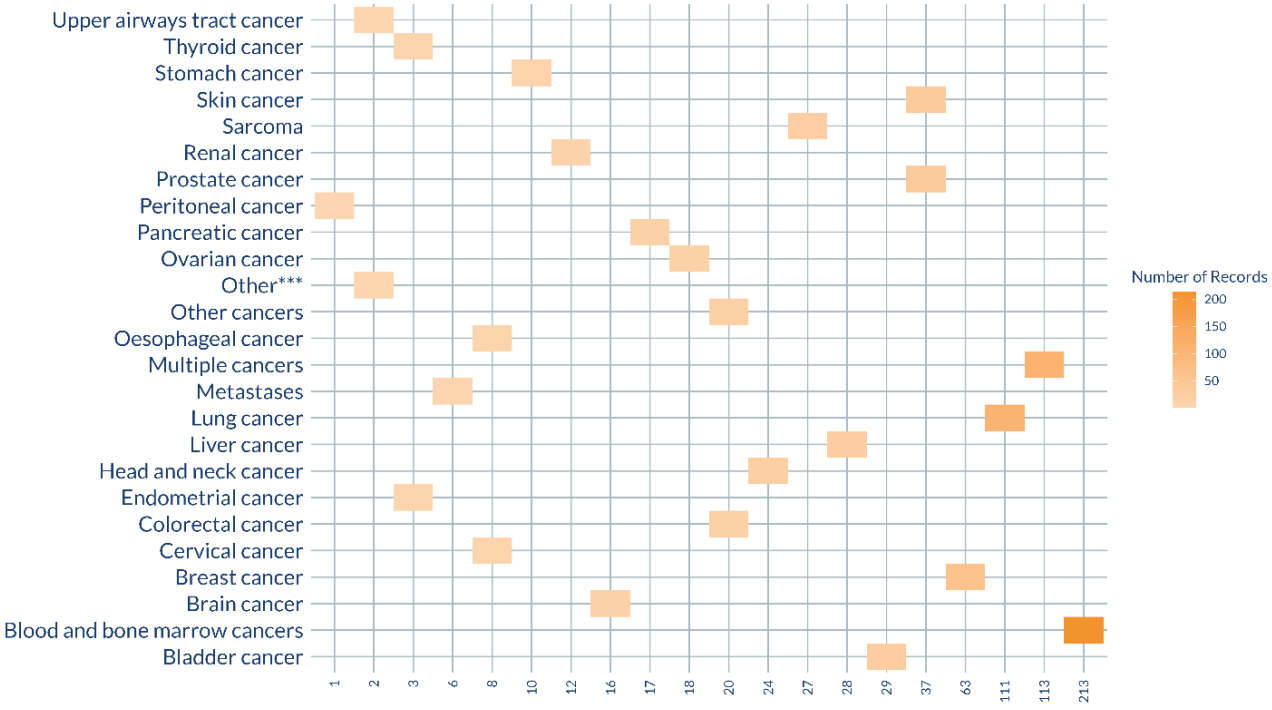


Figure 1. Number of records for cancer and non-cancer indications

A



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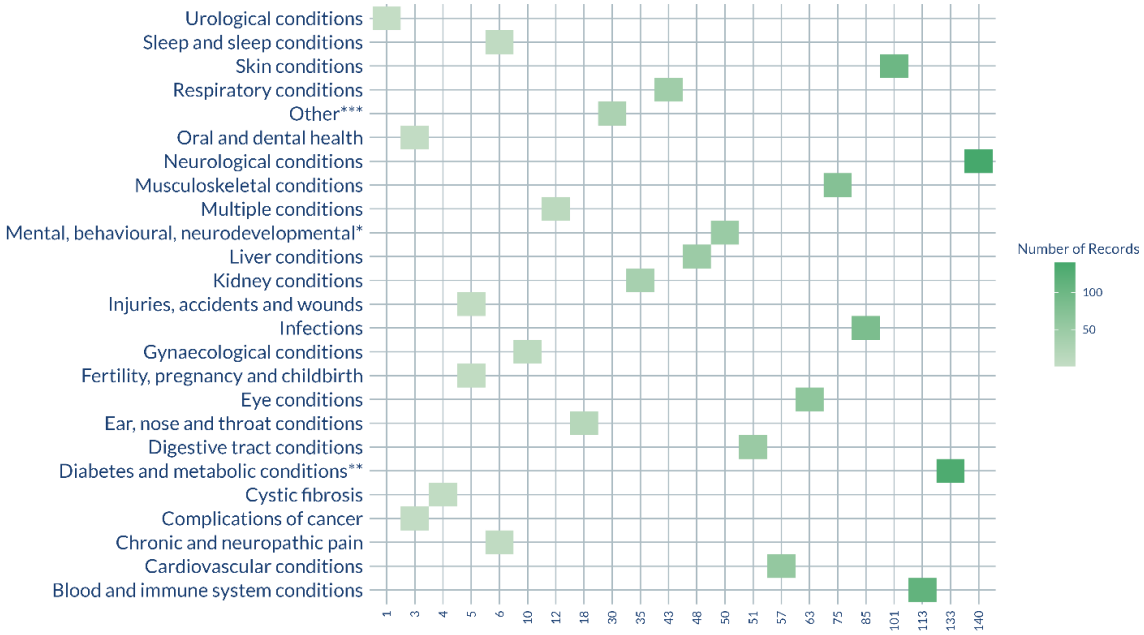


Figure 2. A) Number of records by NICE cancer condition group B) Number of records by NICE non-cancer condition group 3

ii. Indications for female population group

Our analysis showed 108 (5.6%) out of 1,925 records for conditions affecting women's health. Out of these, 92 records were for cancer indications. Breast cancer had the most records (n=63, 58.3%) followed by ovarian cancer (18, 16.7%), cervical cancer (8, 7.4%), and endometrial cancer (3, 2.8%) as shown in figure 3. Non-cancer indications included fertility, eclampsia, infections, postmenopausal vasomotor symptoms, vaginal atrophy, contraception, and female sexual arousal disorder, and comprised of 16 records (15%).

[3] *Mental health, behavioural and neurodevelopmental conditions. ** Diabetes and other endocrinal, nutritional, and metabolic conditions. ***Indications not fitting into the NICE categories

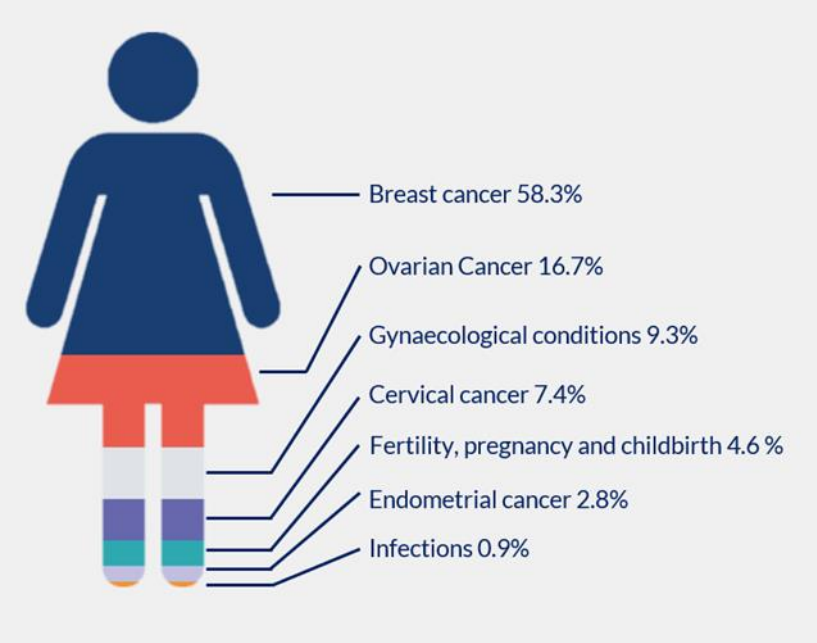


Figure 3. Percentages of all female-only condition records

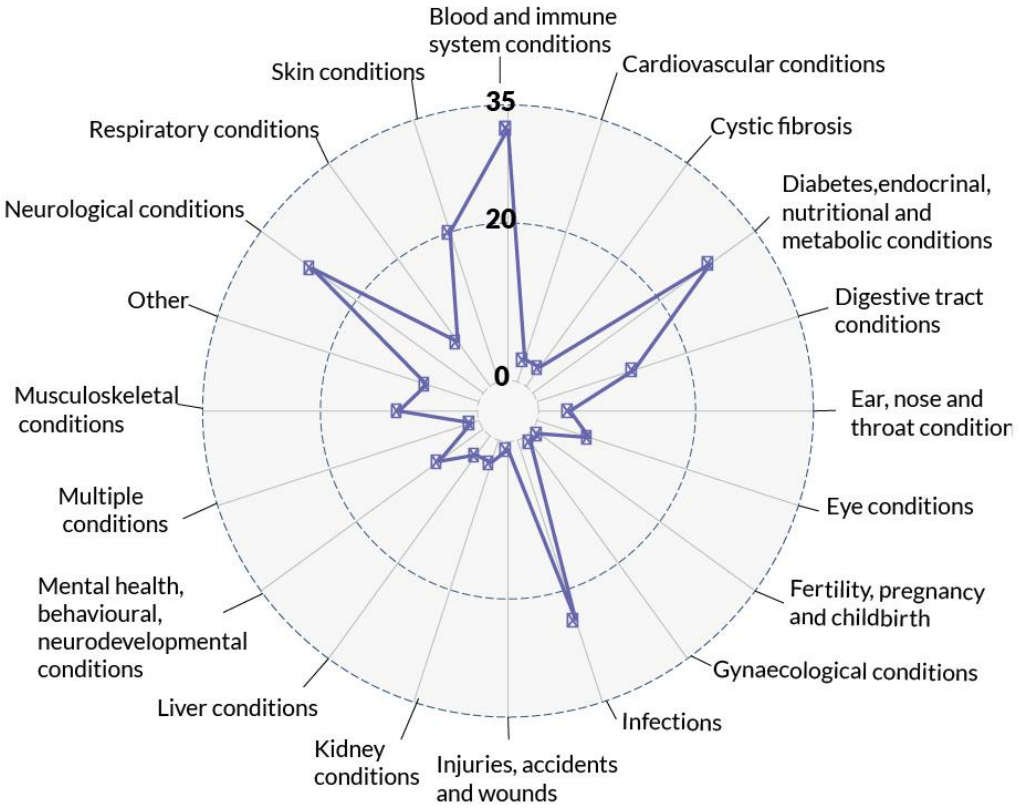
iii. Indications for paediatric population

There were 254 (13%) records which include paediatric population either alone or with adult population. Almost 80% of the records were for non-cancer indications while cancer records made up 20% of the total paediatric records.

Blood and immune system conditions, diabetes and other endocrinal, nutritional and metabolic conditions, and neurological conditions had the highest number of records (Figure 4A). Within blood and immune system conditions, graft vs host disease, sickle cell disease, and primary immunodeficiency, were the most common.

Within cancer records, multiple cancers formed the largest group (Figure 4B). The indications included in this group are solid tumours often targeting a specific subgroup (gene mutation such as NTRK or ALK fusion). Blood and bone marrow cancers form the second largest group with most of the indications being acute or chronic leukaemia.

A


Blood and immune system conditions
Cardiovascular conditions
Cystic fibrosis
Diabetes,endocrinal, nutritional and metabolic conditions
Digestive tract conditions
Ear, nose and throat conditior
Eye conditions
Fertility, pregnancy and childbirth
Gynaecological conditions
Infections
Injuries, accidents and wounds
Kidney conditions
Liver conditions
Mental health, behavioural, neurodevelopmental conditions
Multiple conditions
Musculoskeletal conditions
Other
Neurological conditions
Respiratory conditions
Skin conditions
35
20
0

B

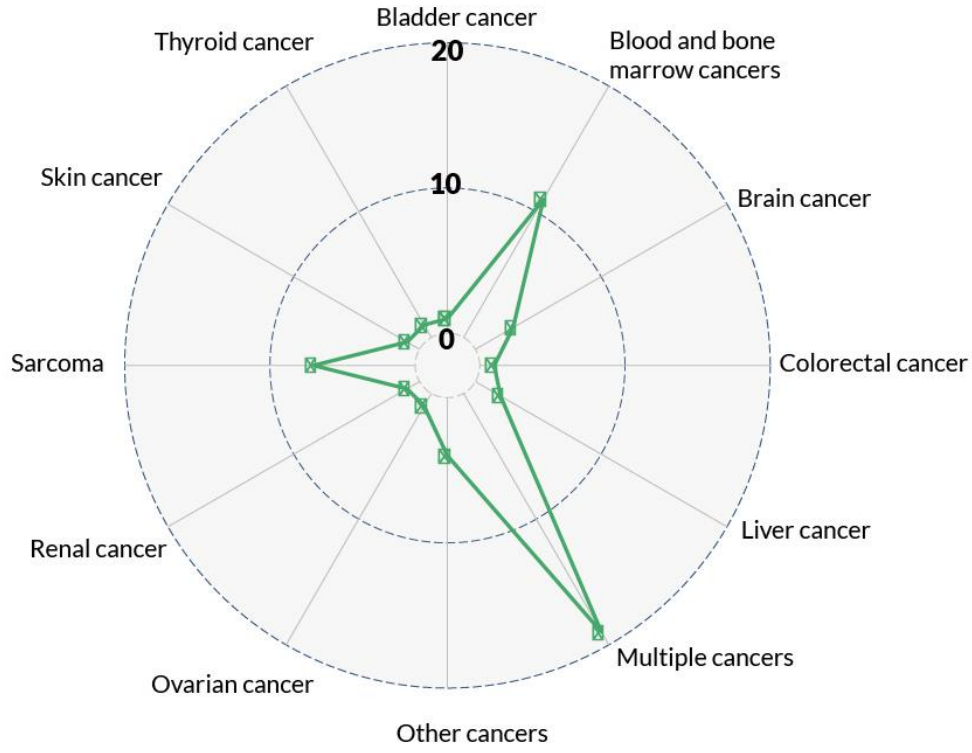


Figure 4. A. Records for paediatric or mixed population by NICE non-cancer condition group B. Records for paediatric or mixed population by NICE cancer condition group[4]

2. Interventions

i. Monotherapies vs combination therapies

Over two-thirds of the 1,925 records were for monotherapies (n=1,294, 67%) while one-third (n=631, 33%) were for combination therapies. Most non-cancer records (n=980, 51%) were for monotherapies while most cancer records (n=514, 27%) were for combination therapies (Figure 5).

[4] 'Other' includes indications that do not fall under any NICE indications. 'Other cancers' includes cancers that do not fit into any NICE indications

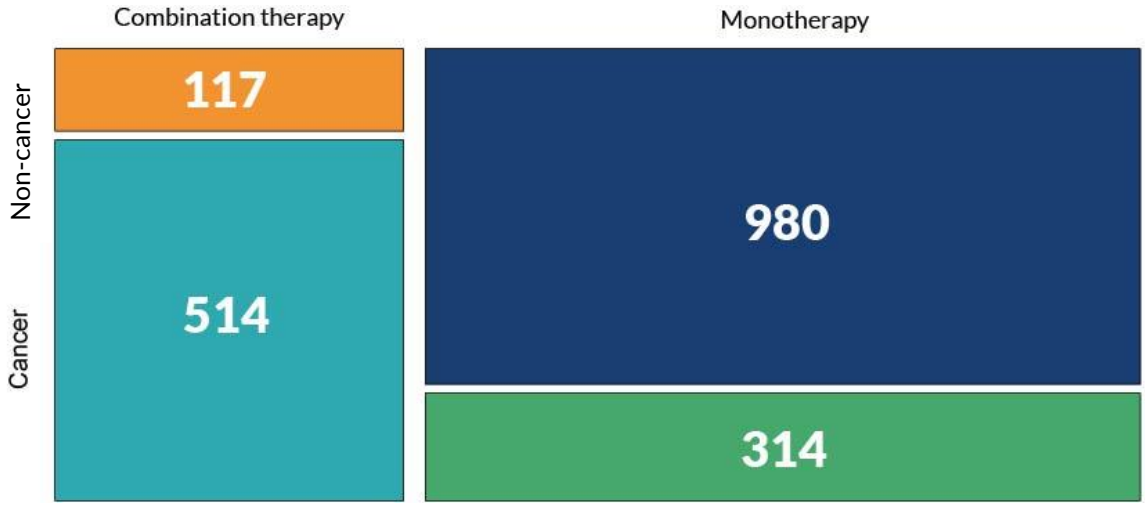


Figure 5. Number of records for monotherapies and combination therapies by type of indication

Blood and bone marrow cancer, lung cancer, and multiple cancers, had the most records where technologies were being investigated as combination therapies. Neurological conditions, diabetes and other endocrinal, nutritional, metabolic condition, and blood and immune system condition had the most records for monotherapies.

ii. Biosimilars

A biosimilar medicine is defined as 'medicine which has been shown not to have any clinically meaningful differences from the originator medicine in terms of quality, safety and efficacy'.[3] Out of 1,925 records, 61 records (3%) were for those that included a biosimilar. We identified 17 unique biosimilars from these 61 records for 17 originator medicines in the 2021 dataset. Most records were for biosimilars for etanercept, and pegfilgrastim (both non cancer) (Figure 6). Most of the records were for non-cancer indications, which included, musculoskeletal conditions, skin conditions, and multiple conditions, and diabetes and other endocrinal,

nutritional, metabolic conditions. For cancer the most common biosimilars were for trastuzumab.

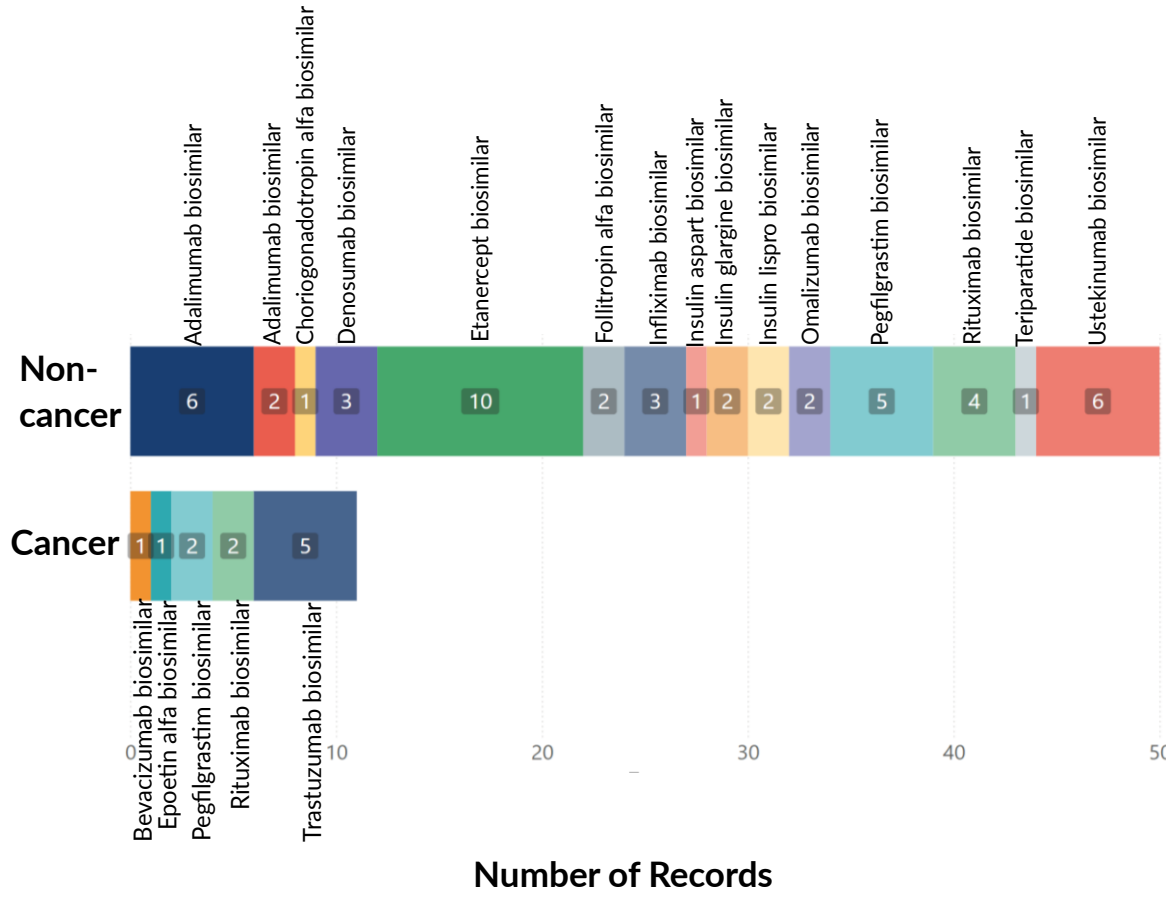


Figure 6. Number of records for biosimilars

iii. Advanced therapy medicinal products

Advanced therapy medicinal products (ATMPs) are defined by the European Medicines Agency as medicines for human use that are based on genes, tissues or cells and include three categories; gene therapy, somatic-cell therapy, and tissue-engineered medicines.[2] The 2021 records on MInD included 12 (0.6%) medicines that fulfilled the criteria for an EMA ATMP (Figure 7).

More than half the ATMPs were cell-based therapies. Two-thirds of all the records were for cancer indications. Blood and bone marrow cancers were the most common cancer indication, most of which were for multiple myeloma. Most common non-cancer indication was blood and immune system condition.

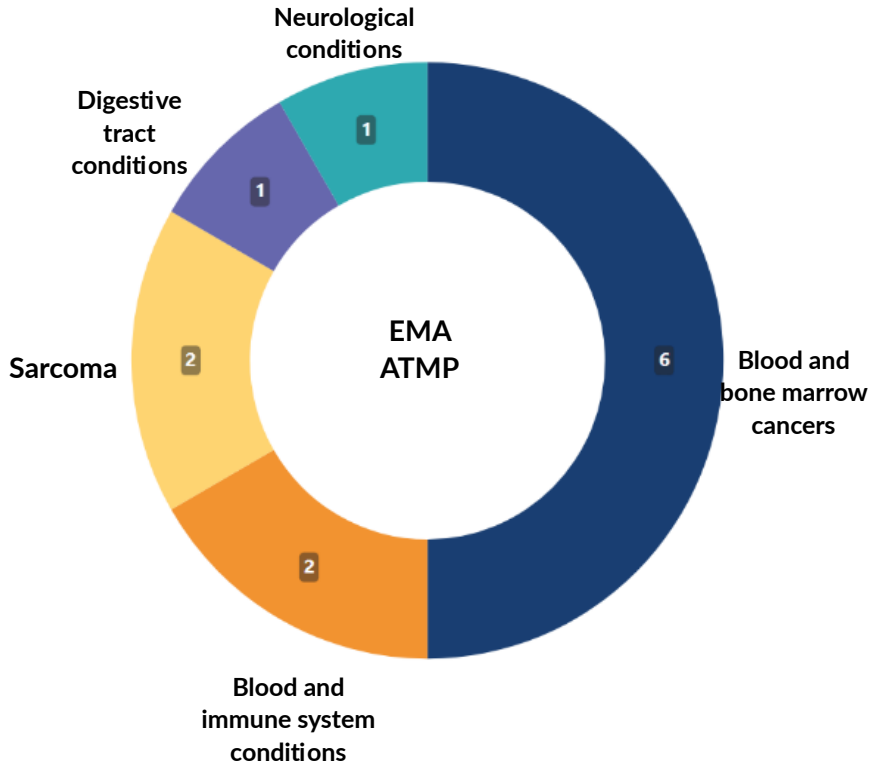


Figure 7. Records containing at least one EMA-classified ATMP

3. Regulatory information

i. Orphan drugs

Orphan drugs as defined by the EMA are medicines that must meet the following criteria:[5]

- Must be intended for the treatment, prevention or diagnosis of a disease that is life-threatening or chronically debilitating;
- The prevalence of the condition in the EU must not be more than 5 in 10,000 or it must be unlikely that marketing of the medicine would generate sufficient returns to justify the investment needed for its development;
- No satisfactory method of diagnosis, prevention or treatment of the condition concerned can be authorised, or, if such a method exists, the medicine must be of significant benefit to those affected by the condition.

In 2021, 157 records (8%) were related to medicines that had orphan drug designations by the EMA. A large majority (111, 71%), of the records were for non-cancer indications while the rest (44, 29%) were for cancer indications (Figure 8). Blood and bone marrow cancers were the most common cancer indications and blood and immune system conditions the most common non-cancer indications for which orphan drugs were in development. Most orphan drugs being used for both cancer and non-cancer indications were in trials as monotherapy (Figure 8).

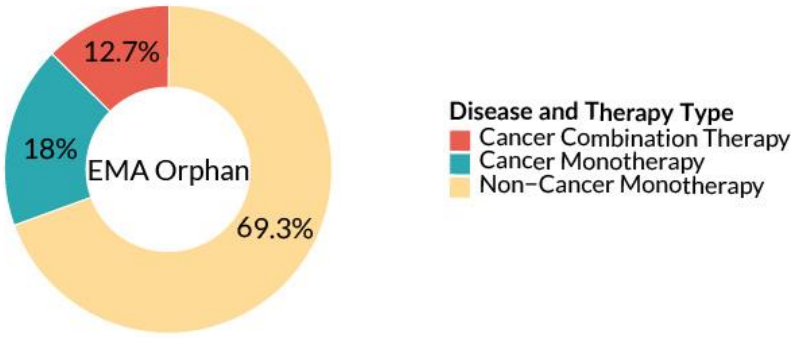


Figure 8. Records with EMA Orphan drug designation by treatment and therapy type

ii. Priority Medicines

Priority Medicines (PRIME) is a voluntary scheme conducted by the EMA to support the development of medicines that address an unmet medical need for which no treatment option exists or where a medicine can offer major therapeutic advantage compared to existing treatments.[6] This allows for an expedited marketing authorisation approval in the EU.

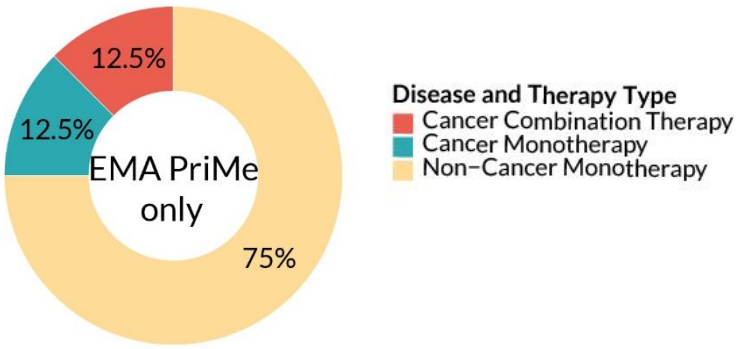


Figure 9. Records with EMA PRIME designation by treatment and therapy type

There were 22 records for 21 medicinal products on MInD for 2021 which were for technologies given PRIME designation (Figure 9). Non-cancer indications were being targeted more for treatment with drugs with PRIME designations (88%), however blood and bone marrow cancers was the most represented single condition. Our analysis found that nearly all drugs with PRIME designations were being investigated as monotherapies.

iii. Promising Innovative Medicines

Promising Innovative Medicines (PIM) designation is intended for treatment, diagnosis, or prevention of a life-threatening or seriously debilitating condition that could potentially address an unmet medical need.[7] It facilitates an application for the Early Access to Medicines Scheme (EAMS) with the Medicines and Healthcare products Regulatory Authority (MHRA) in the UK. Our analysis of the 2021 dataset showed 6 records for medicines with PIM designations (Figure 10), one of which appeared to be a potential ATMP. Most of these records (67%) were for non-cancer indications.

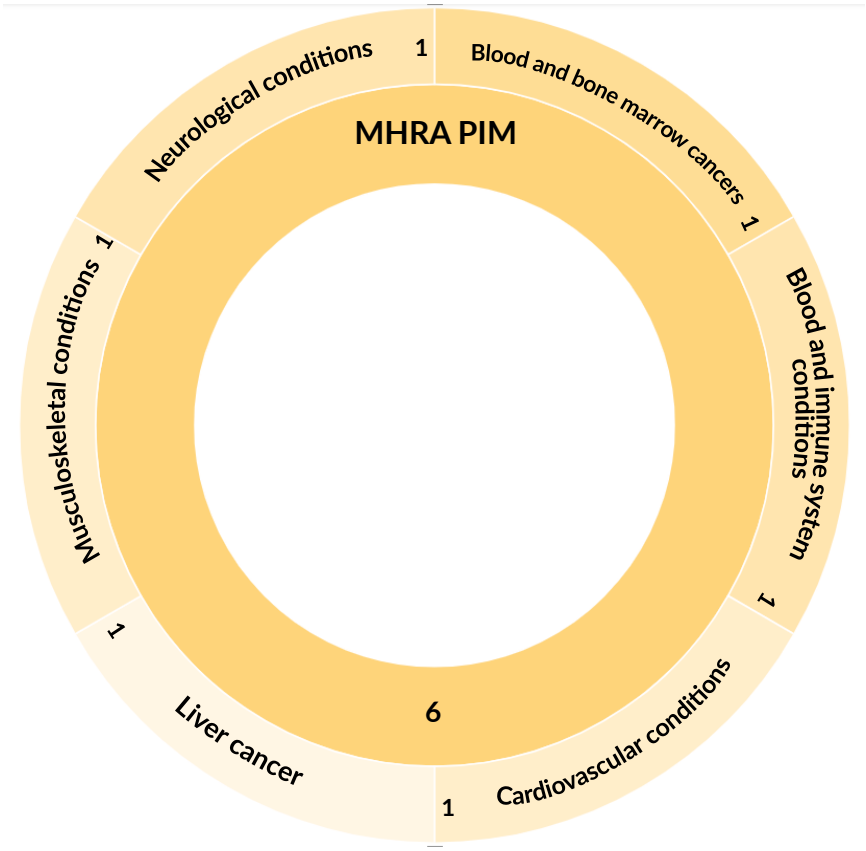


Figure 10. Number of records with PIM designations

iv. Innovation Licensing and Access Pathway

The MHRA introduced the Innovative Licensing and Access Pathway (ILAP) in January 2021. This initiative aims to accelerate the time to market, ultimately facilitating patient access to medicines.[8] In the 2021 MInD data, 4 records included a technology that had received an ILAP (Figure 11). Two of the technologies were monoclonal antibodies. All of the records were for cancer indications. Two records were for lung cancer, one for skin cancer and one for blood and bone marrow cancers.

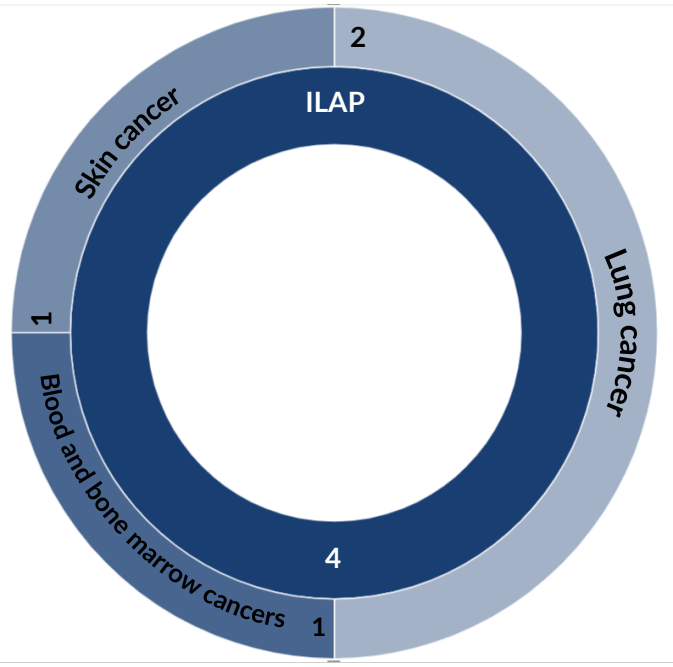


Figure 11. Number of records with ILAP

4. Developers

Figure 12 shows the 15 companies which had the most records linked to them on IO's MInD database in 2021. The majority of records for most companies were related to cancer indications compared to non-cancer indications. Figure 13 shows the companies with the highest number of cancer and non-cancer records on MInD database in 2021 while figure 14 shows the companies with the highest number of records for most common cancer indications in the UK.

We also examined which companies were developing orphan, PRIME, PIM, ILAP designated drugs. We found that Pfizer Ltd UK had the most records (n= 8, 0.4%), along with Novartis (n= 8, 0.4%), Janssen (n= 5, 0.25%).

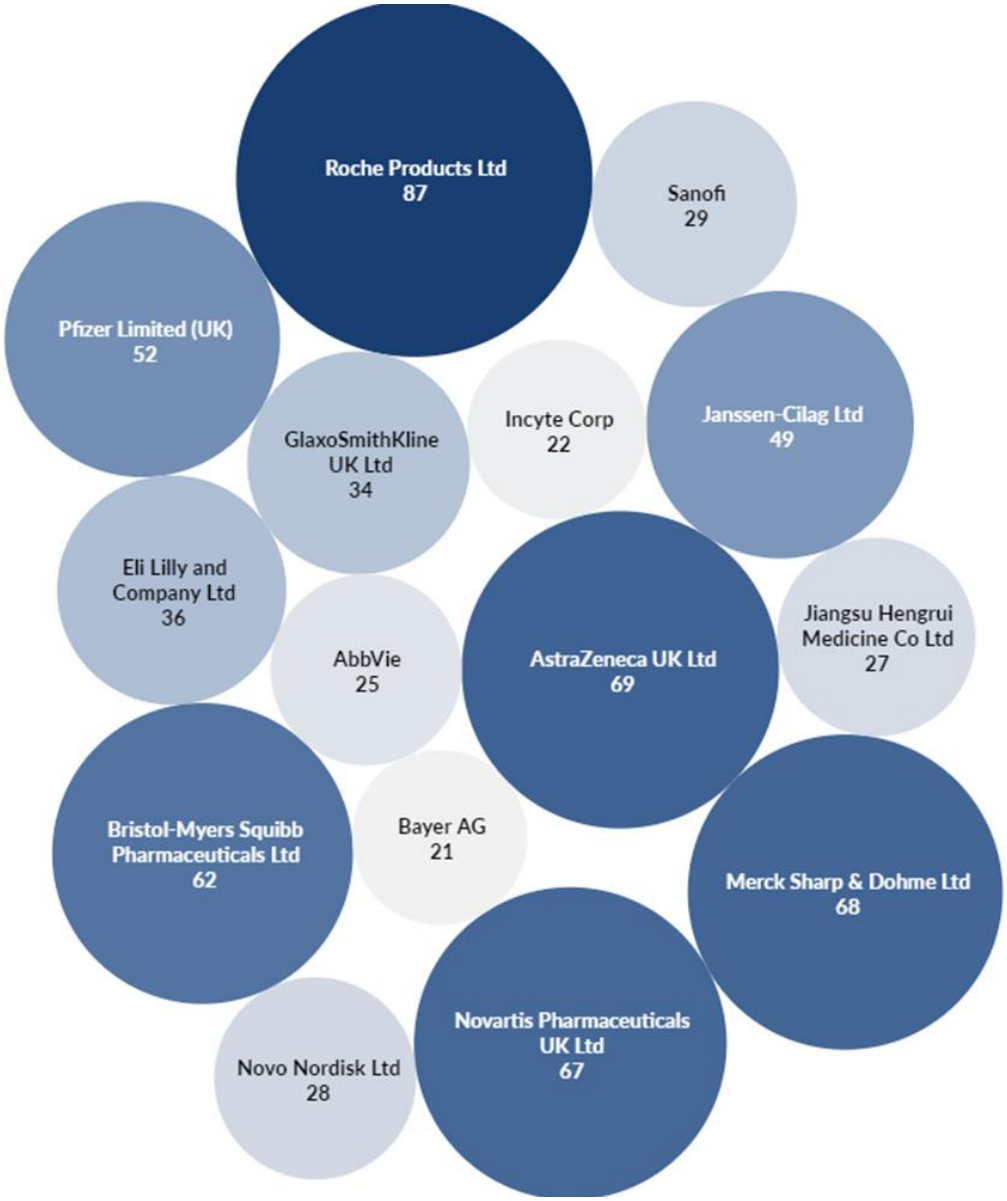


Figure 12. Companies with the most records on MInD database in 2021

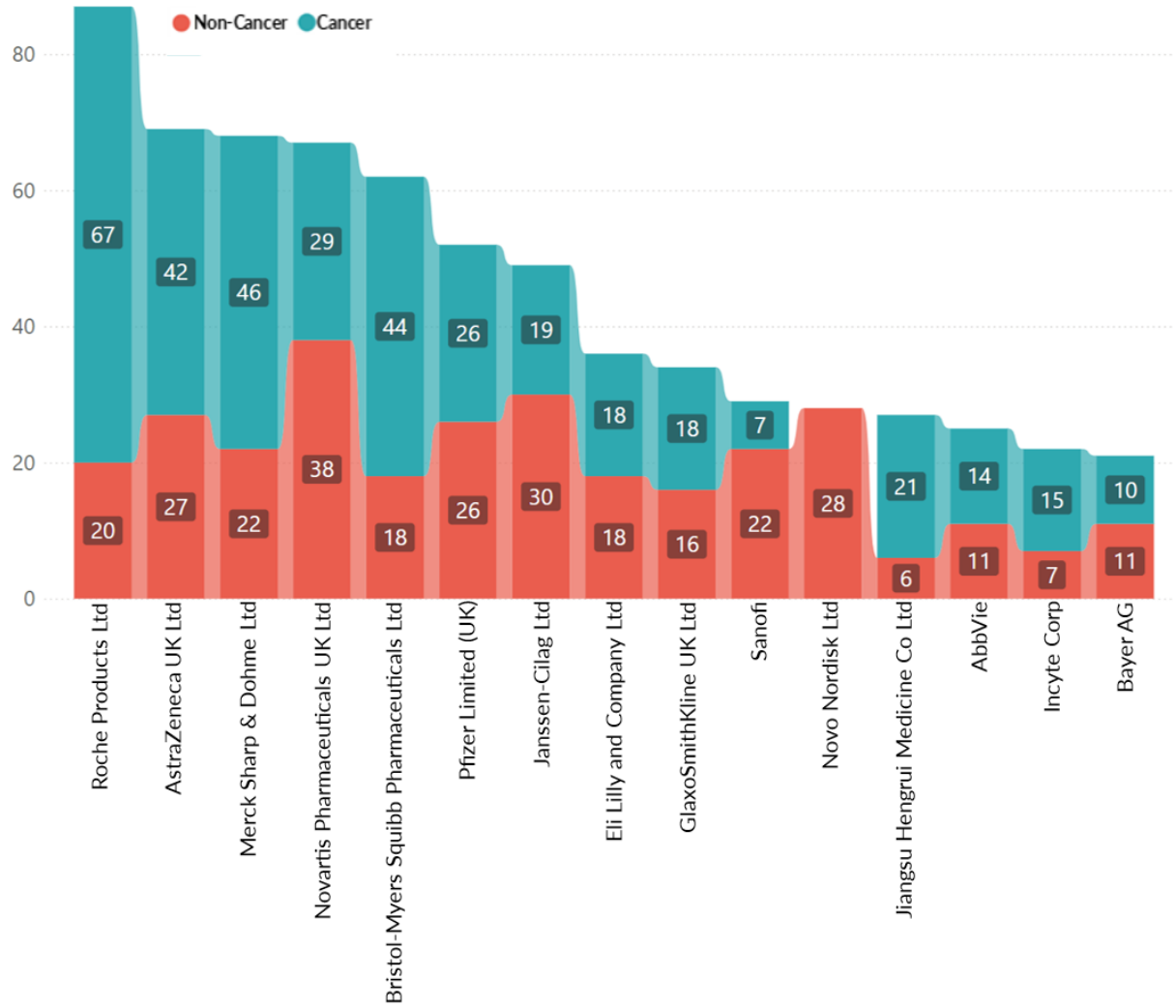


Figure 13. Companies with number of cancer and non-cancer records on MInD database in 2021

Company	Blood and bone marrow cancers	Breast cancer	Lung cancer	Prostate cancer	Skin cancer
Bayer AG	1			2	
Sanofi	6				
GlaxoSmithKline UK Ltd	5	3	2		
AbbVie	10		1		
Incyte Corp	11				1
Jiangsu Hengrui Medicine ..	2		7	3	
Eli Lilly and Company Ltd	4	5	3	1	
Janssen-Cilag Ltd	11		3	2	
Merck Sharp & Dohme Ltd	4	1	7	3	3
Novartis Pharmaceuticals ..	10	3	4	1	1
Pfizer Limited (UK)	7	6	3	2	3
AstraZeneca UK Ltd	10	5	11	1	
Bristol-Myers Squibb Phar..	16		7	2	6
Roche Products Ltd	10	10	10		2

Figure 14. Companies with the highest number of records for common cancers in UK

Conclusion

This report presents a snapshot of the new or updated records on IO's MInD database in 2021. We analysed the technology records based on indications, interventions, regulatory information, and sponsor information. Most non-cancer records were for monotherapies while most cancer technology records were for combination therapies. Two thirds of the ATMP records were for cancer indications while most biosimilar records were for non-cancer indications.

Around 5.6% of the total records were specifically relevant to the female population. About 13% were relevant to the paediatric and mixed population. %). Nearly 39% of the 2021 MInD records were tagged as rare diseases. Most orphan drug designations and PRIME designations in 2021 were for non-cancer indications, however, most of PIM designations were for non-cancer indications.

Future work

This report is the second annual report produced by IO. We aim to produce regular reports giving a picture of innovation in the sphere of clinical development for medicines in the

previous year. Going forward IO will also work to develop reporting on classes of drugs (to capture development of new molecular targets); clinical development in specific indications identified as areas of unmet need. We will also consider developments in the horizon scanning landscape over time, allowing the detection of trends in medicines development.

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Acknowledgements and Disclaimers

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