Medicines Horizon Scanning Annual Report 2020

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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 IO medicines horizon scanning team's horizon scanning activity in 2020. It summarises the horizon scanning strategy used to identify newly created or updated records within 2020. It also gives 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 based on the themes and patterns identified.Methods [ensure to use Heading 1 style]

Methods

1. Medicines Innovation Database

The IO has developed a bespoke, internal, 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 websites, press releases, direct engagement with companies)

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 falls within the scope of the IO medicines horizon scanning if it fulfils the following criteria:

- Medicinal products including 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[a] 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 2020. 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 2020 landscape of medicines in remit for IO

Our analysis showed that from a total of 2,266 records, 1,938 were created and 338 were updated in 2020 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 the specific populations involved.

1. Indications

i. Indications for cancer and non-cancer

An close number of cancer and non-cancer records were observed in the 2020 data on MInD (Figure 1). Figures 2 and 3 show the number of MInD records for cancer (n=1,114, 49%) and non-cancer (n=1,152, 51%) indications classified by NICE categories.

[a] 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

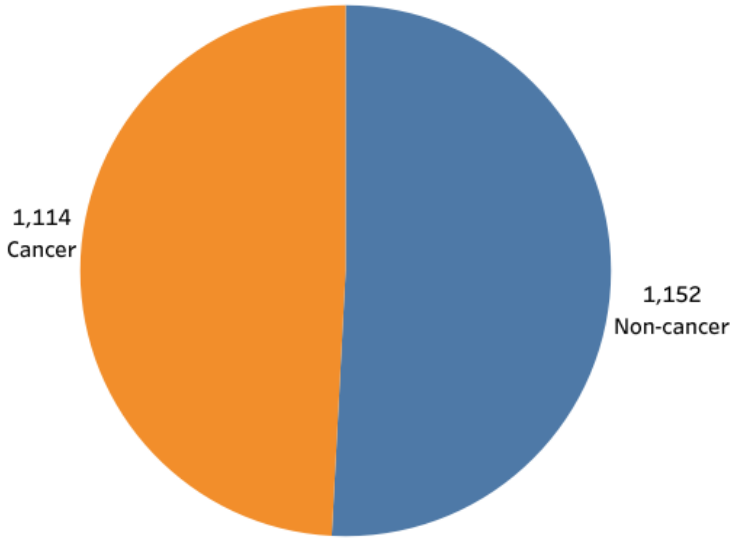

Figure 1. Number of technology records for cancer and non-cancer indications.

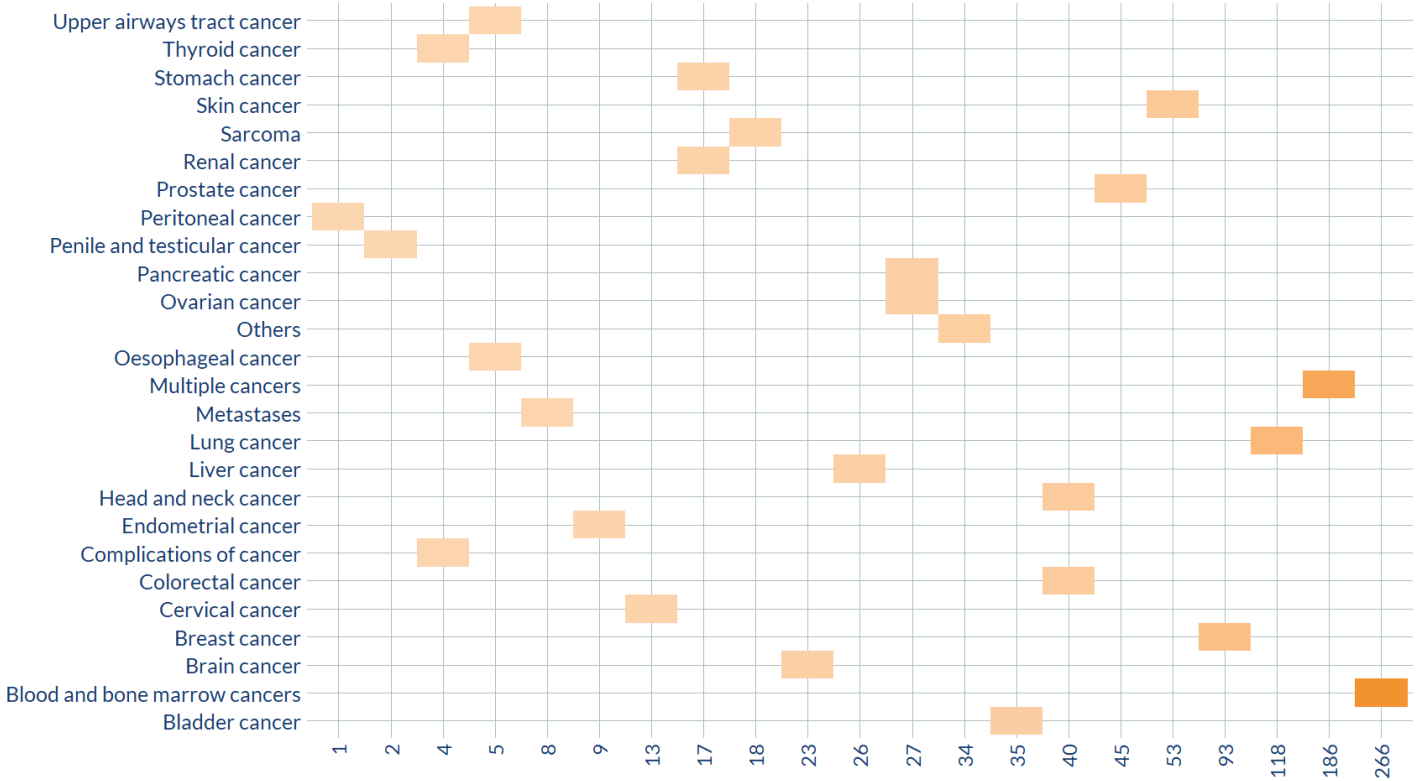

Figure 2. Number of records by NICE cancer condition group[b]

[b] Within the figure we have included an 'Other' category which represents indications that do not fit into a NICE cancer category such as neuroblastoma

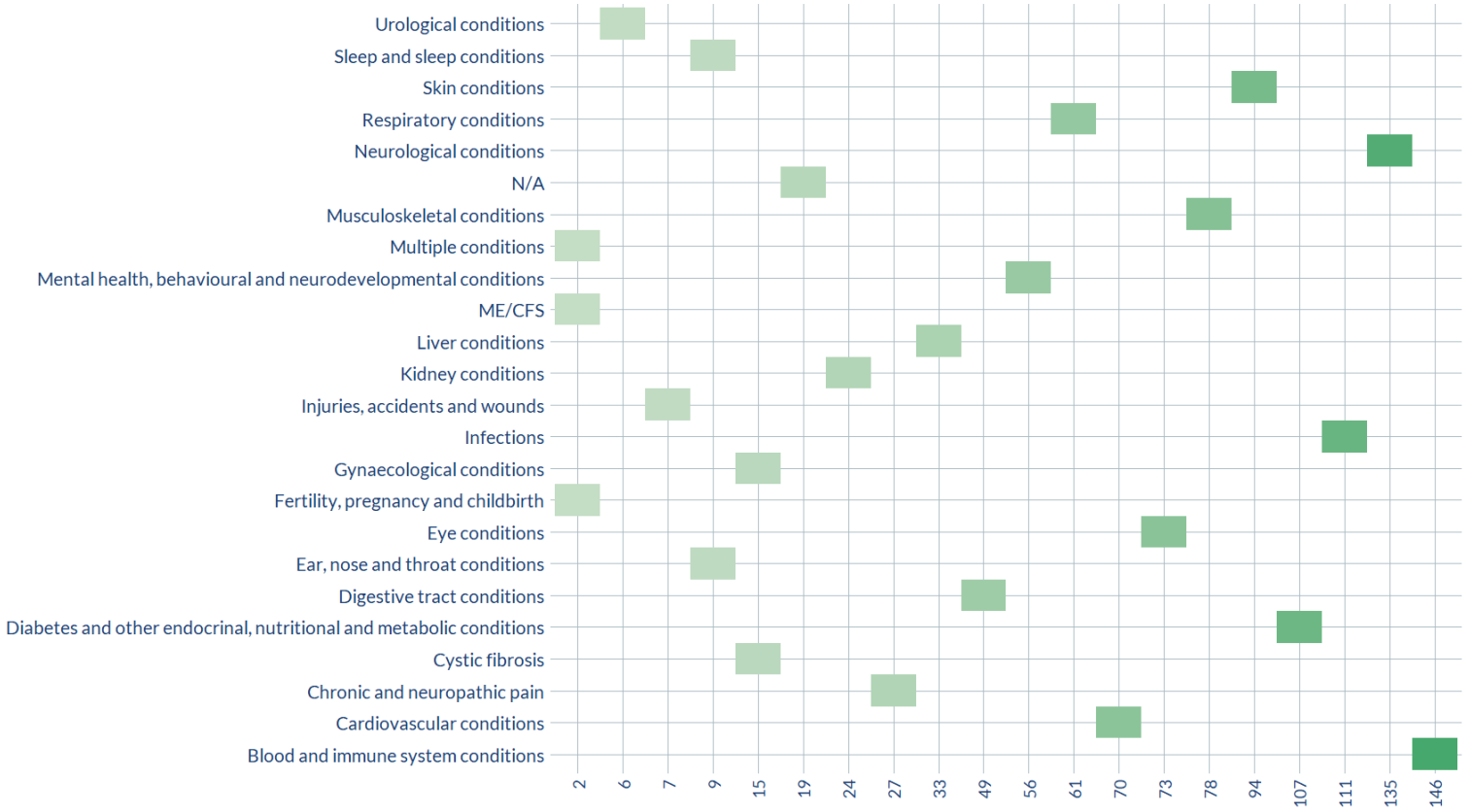

Figure 3. Number of records by NICE non-cancer condition group[c]

[c] Within the figure we have included an 'N/A' category which represents indications that do not fit into a NICE non-cancer indications such as anaesthesia

ii. Indications for female population group

Our analysis showed 146 (6%) records that included trials investigating medicines for conditions specifically affecting women's health. Most (91%) of the clinical trials for female population focussed on cancer indications. Breast cancer had the most records (n=93, 64%) followed by ovarian cancer (n=27, 18%), cervical cancer (n=13, 9%), and endometrial cancer (n=9, 6%) as shown in figure 4. Non-cancer indications included fertility, eclampsia, postmenopausal vasomotor symptoms, vaginal atrophy, contraception, and female sexual arousal disorder, and comprised of 10% of the records.

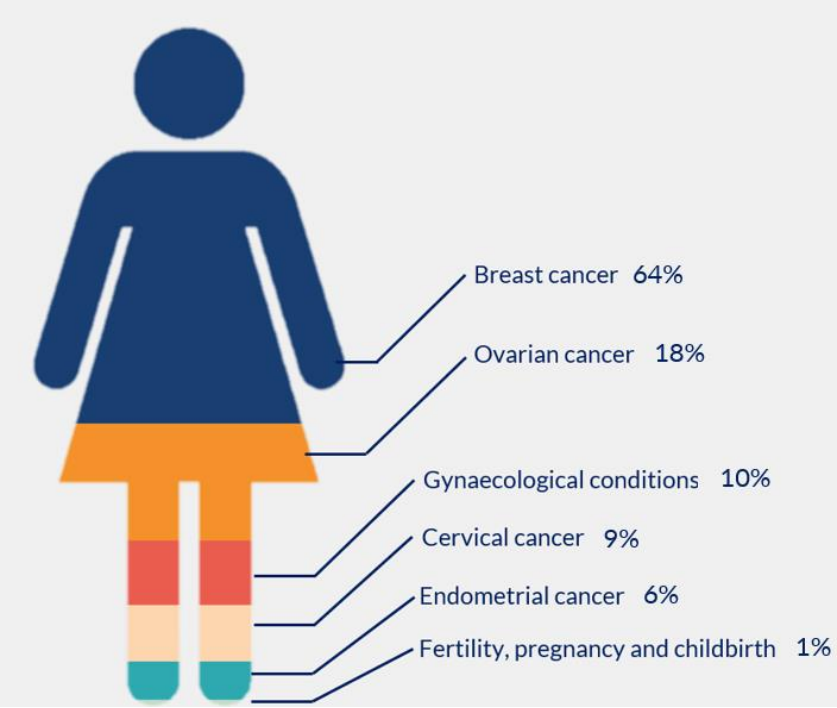

Figure 4. Percentages of all female-only condition records

iii. Indications for paediatric conditions

We assessed the number of records that included clinical trials which enrolled children or mixed populations of children and adults (Figure 5). There were 203 relevant records found, across a wide range of indications. Blood and bone marrow cancers and blood and immune system conditions were the most common cancer and non-cancer indications respectively.

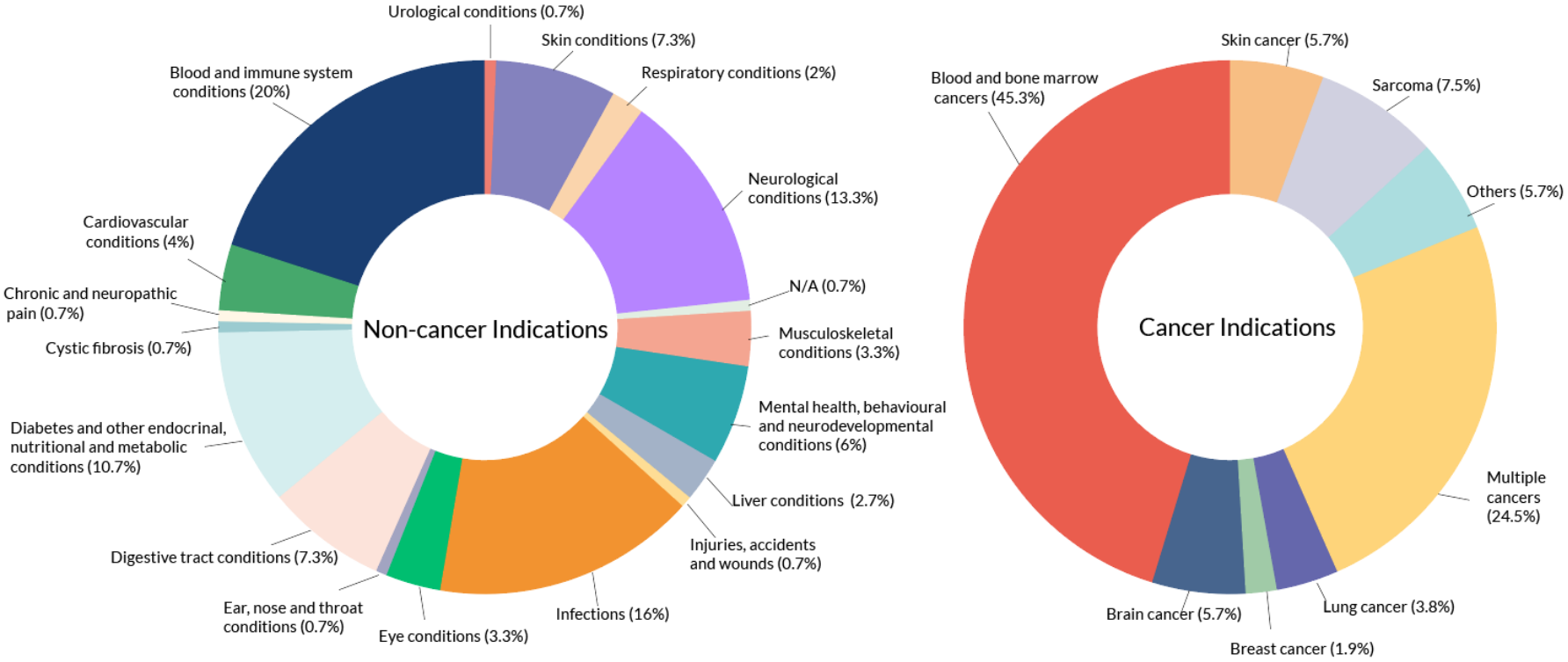

Figure 5. Records for paediatric or mixed populations by NICE non-cancer and cancer conditions Number of cancer and non-cancer records with trials investigating conditions in paediatric or mixed populations[d]

[d] The category of 'N/A' includes conditions that don't fit well into the NICE indication categories e.g. anaesthesia or idiopathic short stature. 'Others' includes cancer indications that do not fit into any NICE caner categories such as retinoblastoma

2. Interventions

i. Monotherapies vs combination therapies

Out of 2,266 records, 62% (n=1,408) were for those where the medicine was being used as a monotherapy while 38% (n=858) were for combination therapies (Figure 6). Most non-cancer records (n=1,024, 45%) were for monotherapies while most cancer records (n=732, 32%) were for combination therapies.

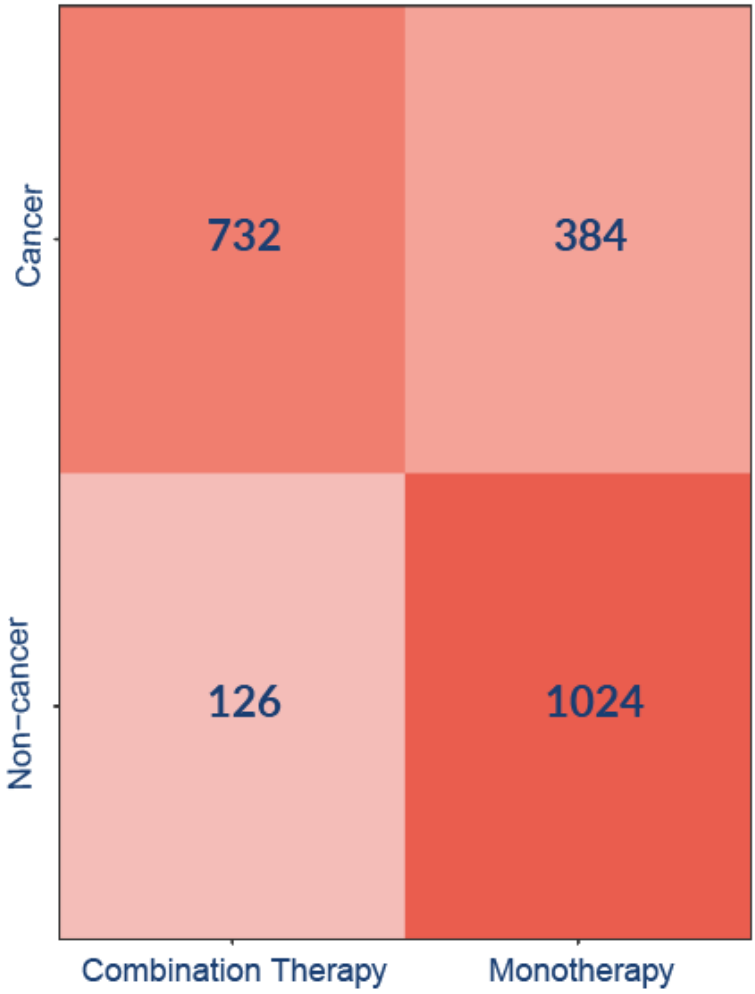

Figure 6. Number of records for monotherapies and combination therapies by type of indication

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'.[5] In 2020, 16 MInD records consisted of biosimilar medicines. The originator medicine for nearly all the biosimilars was either a monoclonal antibody or a tumour necrosis factor inhibitor. Figure 7 shows the main indications being investigated for biosimilars. Eye conditions were the most investigated conditions for biosimilar medicines; all of these were for age-related macular degeneration.

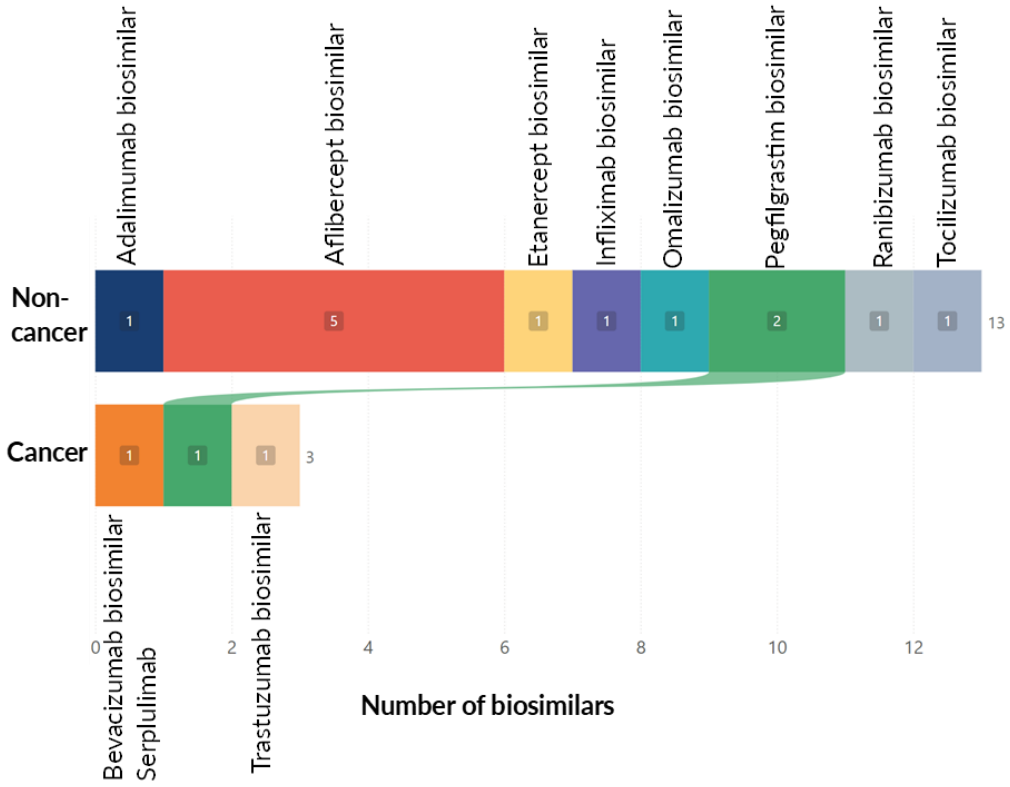

Figure 7. Number of records for biosimilar medicines

iii. Advanced Therapy Medicinal Products

Advanced Therapy Medicinal Products (ATMPs) are defined by the European Medicines Agency (EMA) 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.[4] The 2020 records on MInD included 24 medicines that fulfilled the criteria for an EMA ATMP. Figure 8 shows the indications that had the most ATMP records against them.

Cell therapy products formed a majority of the ATMPs in the 2020 dataset. Most of the ATMPs were being investigated as monotherapies. Those that were being investigated as combination therapies were in trials with monoclonal antibodies, protein kinase inhibitors, or another ATMP.

Blood and bone marrow cancers were the most common indications investigated. Within these, multiple myelomas and B-cell lymphomas were the most common blood cancers. Neurological conditions were the most common non-cancer indications. within which spinal muscular dystrophy and metachromatic leukodystrophy were the most investigated.

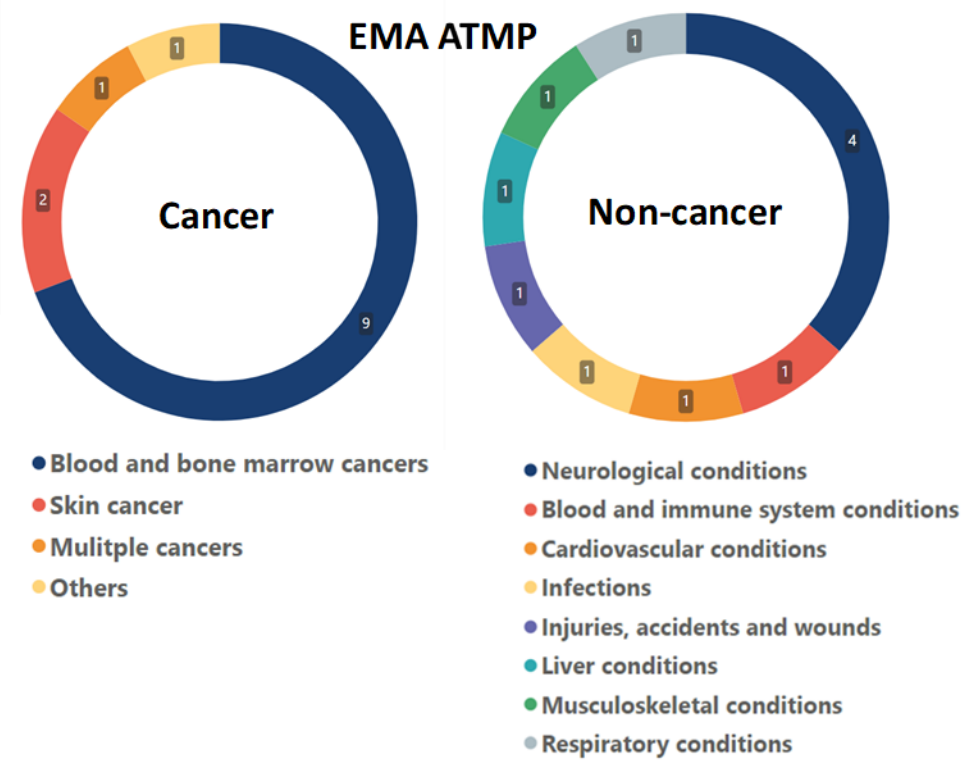

Figure 8. Records containing at least one EMA-classified ATMP by type of indication[e]

3. Regulatory information

i. Orphan drugs

Orphan drugs as defined by the EMA are medicines that must meet the following criteria:[6]

- 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;

[e] Others includes cancer indications that do not fit into NICE cancer categories such as neuroblastoma

- 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 2020, 179 records were related to medicines that had orphan drug designations by the EMA. 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 non-cancer indications were in trials as monotherapy, conversely those for cancer were largely given in combination with other medicines (Figure 9).

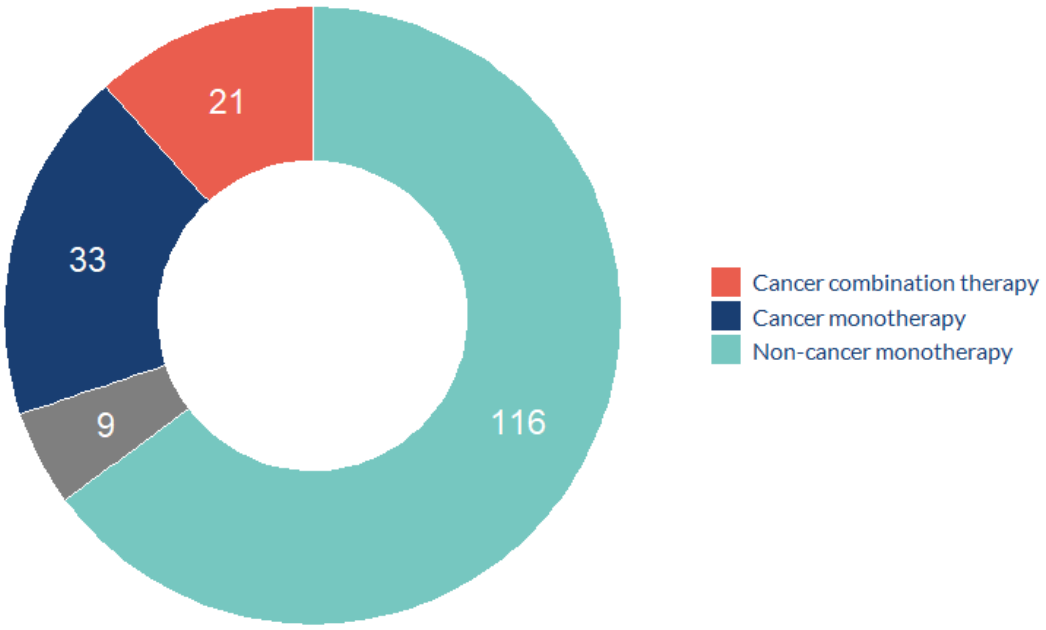

Figure 9. Records for medicines with orphan drug designation.

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.[7] This allows for an expedited marketing authorisation approval in the EU. A total of 29 records in the 2020 MInD dataset were for technologies given PRIME designation (Figure 10). Our analysis found that nearly all drugs with PRIME designations were being investigated

as monotherapies. Non-cancer indications were being targeted more for treatment with drugs with PRIME designations.

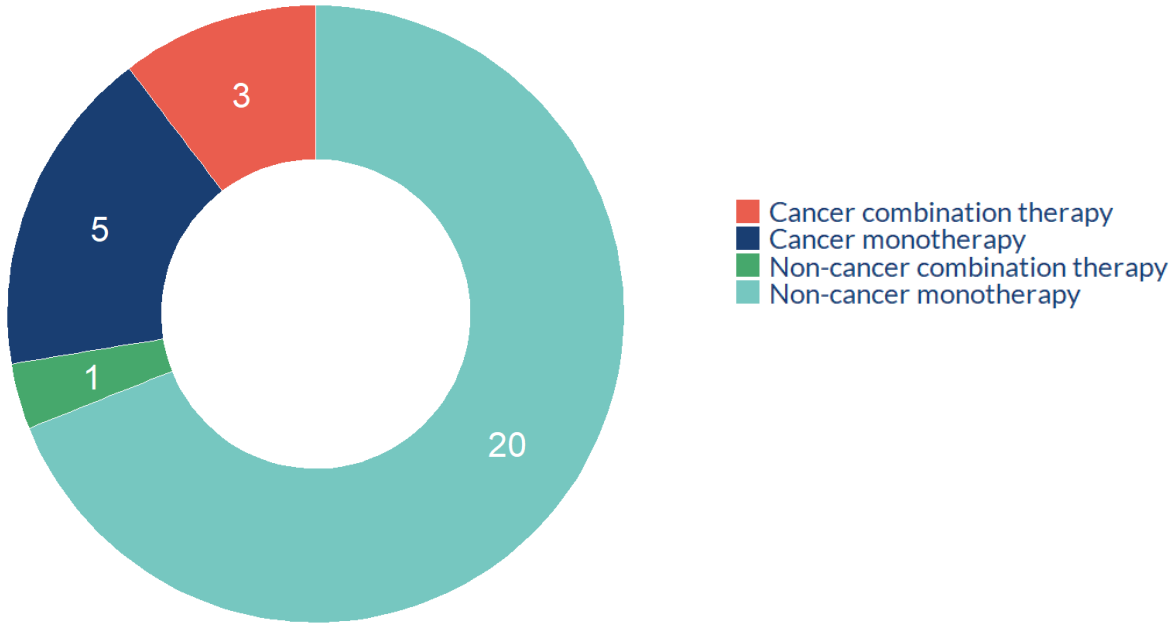

Figure 10. Types of technologies in records with PRIME designations

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.[8] 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 2020 dataset showed seven records for medicines with PIM designations, most of which were for cancer indications (Figure 11).

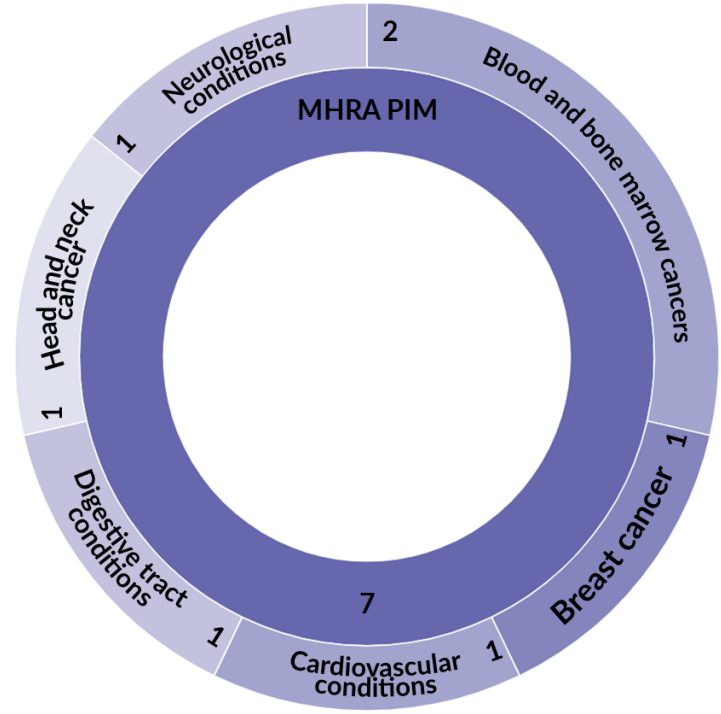

Figure 11. Number of records with PIM designations.

4. Sponsors

Figure 12 shows the 15 companies which had the most records linked to them on IO's MInD database in 2020. The majority of records for most companies related to cancer indications compared to non-cancer indications. Figure 13 shows the companies with the most records against the most common cancers in the UK.

We also examined which companies were developing orphan, PRIME, and PIM, designated drugs. We found that Novartis had the most records (n= 7, 0.3%), followed by Vertex (n= 5, 0.2%), and Janssen (n= 5, 0.2%).

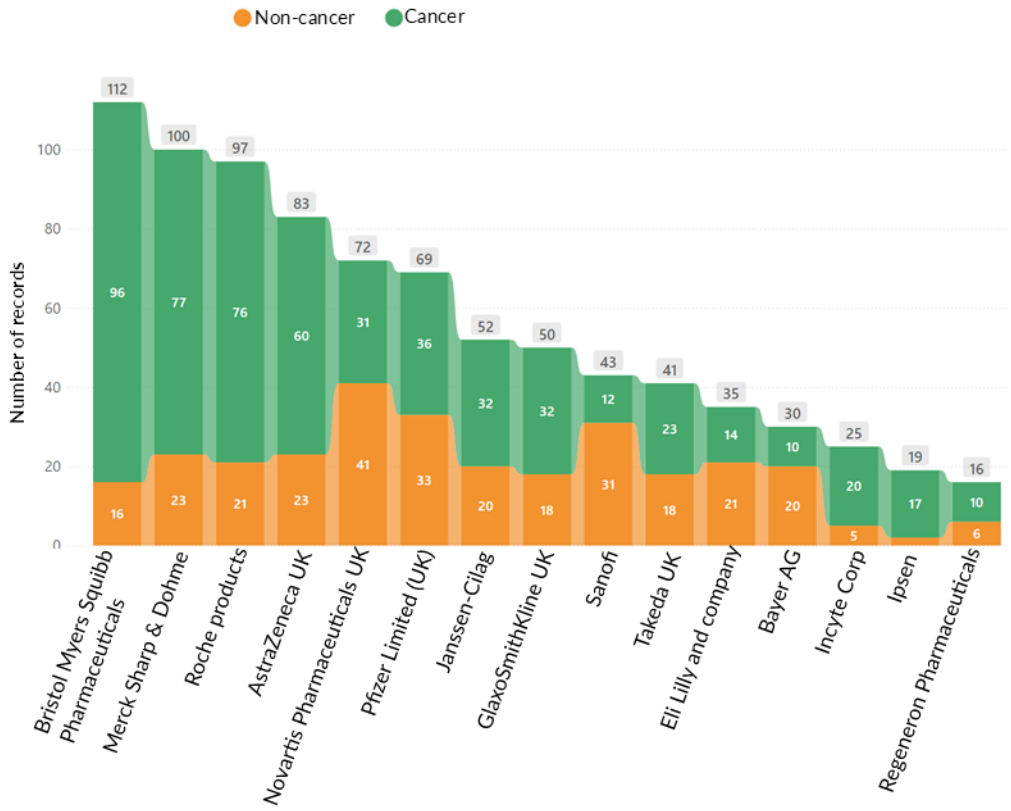

Figure 12. Companies with the most records on MInD database in 2020.

Company	Indications				
	Blood and bone marrow cancers	Breast cancer	Lung cancer	Prostate cancer	Skin cancer
AbbVie	9	2	1		
Amgen Ltd	11				2
AstraZeneca UK Ltd	5	5	16	6	
Bayer AG	1	1	1	3	
Bristol-Myers Squibb Pharmaceuticals ..	19	5	4	1	6
Eli Lilly and Company Ltd		3	3	1	
GlaxoSmithKline UK Ltd	6	3	2		2
Incyte Corp	4		2		
Janssen-Cilag Ltd	17		2	12	
Merck Sharp & Dohme Ltd	9	7	12	3	6
Novartis Pharmaceuticals UK Ltd	7	4	6		3
Pfizer Limited (UK)	8	7	5	1	3
Roche Products Ltd	14	15	19	2	2
Sanofi	3	3	4		
Takeda UK Ltd	16		2		

Figure 13. 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 2020. We analysed the technology records base on indications, interventions, regulatory information, and sponsor information. Most non-cancer records were for monotherapies while most cancer technology records were for combination therapies. A little over half the ATMP records were for cancer indications, while most biosimilar records were for non-cancer indications. Around 6% of the total records were specifically relevant to the female population while 9% were relevant to children. Most orphan drug designations and PRIME designations were for non-cancer indications while most PIM designations were for cancer indications. Bristol-Myers Squibb Pharmaceuticals Ltd, Merck Sharp and Dohme Ltd, and Roche Products Ltd, were the pharmaceutical companies with the most records in the 2020 dataset on the MInD database.

Future work

This is the first annual report produced by the IO. We aim to produce annual reports to present a snapshot of innovation in the sphere of clinical development for medicines in the previous year. Going forward we will also work to develop reporting on classes of drugs (to capture development of new molecular targets) and clinical development in specific indications identified as areas of unmet need. We will also consider developments in the horizon scanning landscape and methods over time, allowing the detection of trends in medicines development and mapping of improvement in signal detection.

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Acknowledgements and Disclaimers

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