Medicines Horizon Scanning Annual Report 2024

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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 most established 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 patient 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 several ad hoc scans to stakeholders such the NHS and IRLSS group.

Aim

This report provides an overview of the MHS team's horizon scanning activity in 2024. It provides: a summary of the horizon scanning strategy devised to identify newly created or updated[1] records within 2024; 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 sponsors; 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)

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

- Soft intel (news/media)
- Pharma intel (company website, 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 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, Australia, Japan or Singapore

3. **Search Strategy for this report**:

The MInD was queried to identify newly created records and updated records from 1st January – 31st December 2024. 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 2024 landscape of medicines in remit for IO

Our analysis showed that from a total of 1,916 technology records, 1,619 new records were created and 297 were updated in 2024 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

Of the 1,916 records created and updated on MInD in 2024, there were 758 (40%) cancer records, and 1,158 (60%) non-cancer records. Figure 1 show the number of records with indications classified as cancer by NICE condition categories.[1] The most frequent cancer categories were blood and bone marrow cancers (n=180, 24%), multiple cancers (n=142, 19%), lung cancer (n=86, 11%), breast cancer (n=53, 7%) and prostate cancer (n=38, 5%).

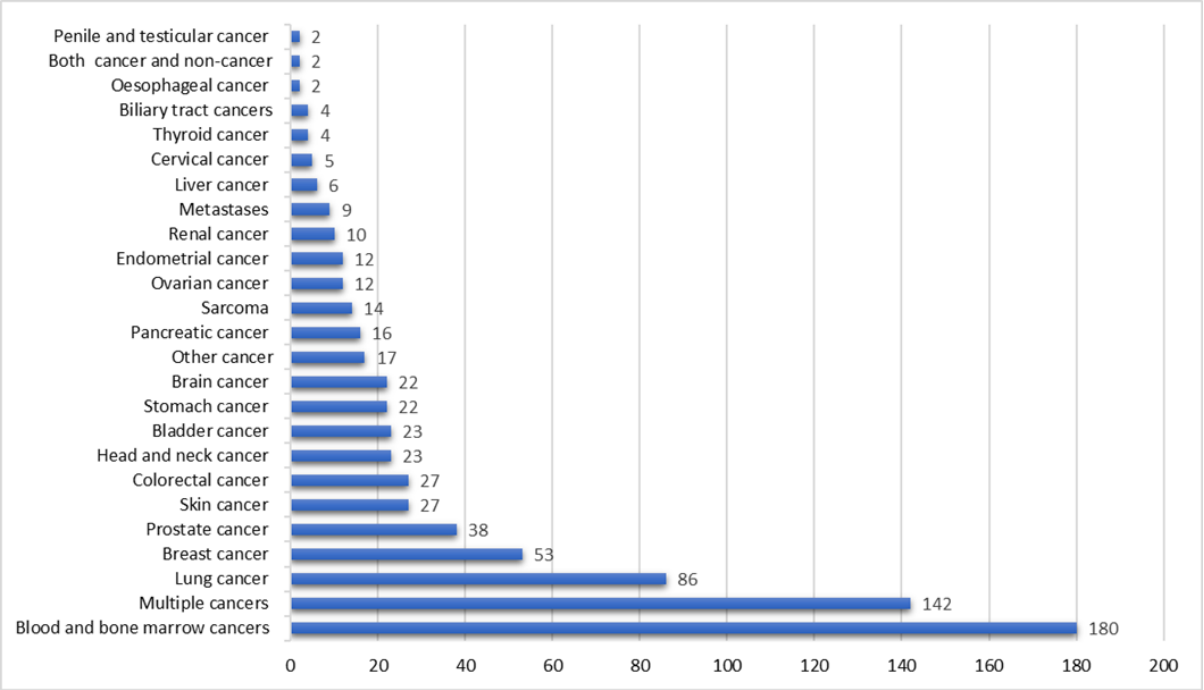

Figure 1. Number of cancer records by NICE condition categories[2]

Figure 2 shows the number of records with indications classified as non-cancer by NICE condition categories.[3] The most common non-cancer categories in these records were neurological conditions (n=139, 12%), blood and immune system conditions (n=134, 12%), diabetes and other endocrinal, nutritional and metabolic conditions (n=121, 10%), skin conditions (n=117, 10%) and infections (n=114, 10%).

[2] Within the figure we have included an 'other cancer' category which represents cancer records that that did not fall into any NICE category

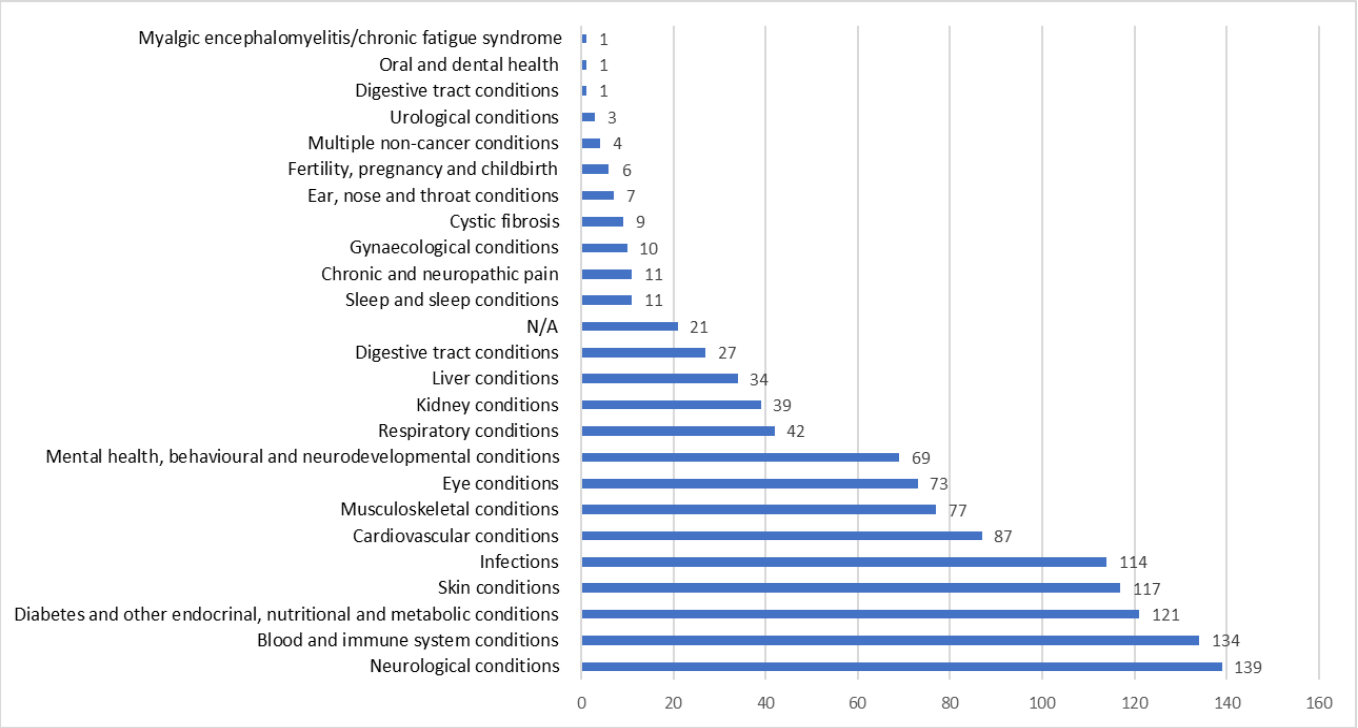

Figure 2. Number of records by NICE non-cancer condition categories[3]

ii. Indication for female population group

We identified 103 (5% of total) records created and updated with indications affecting only the female population. Of these records, 83 (81%) were for indications classified as cancer, and 20 (19%) for non-cancer indications by NICE condition categories. The most common cancer categories in these records were breast cancer (n=53, 52%), ovarian cancer (n=12, 11%) and endometrial cancer (n=12, 11%). Gynaecological conditions (n=10, 10%) and conditions affecting fertility, pregnancy and childbirth (n=6, 6%) were the most frequent non-cancer indications, as shown in Figure 3.

[3] The category of N/A includes conditions that do not fit well into the other indication categories e.g. anaesthesia

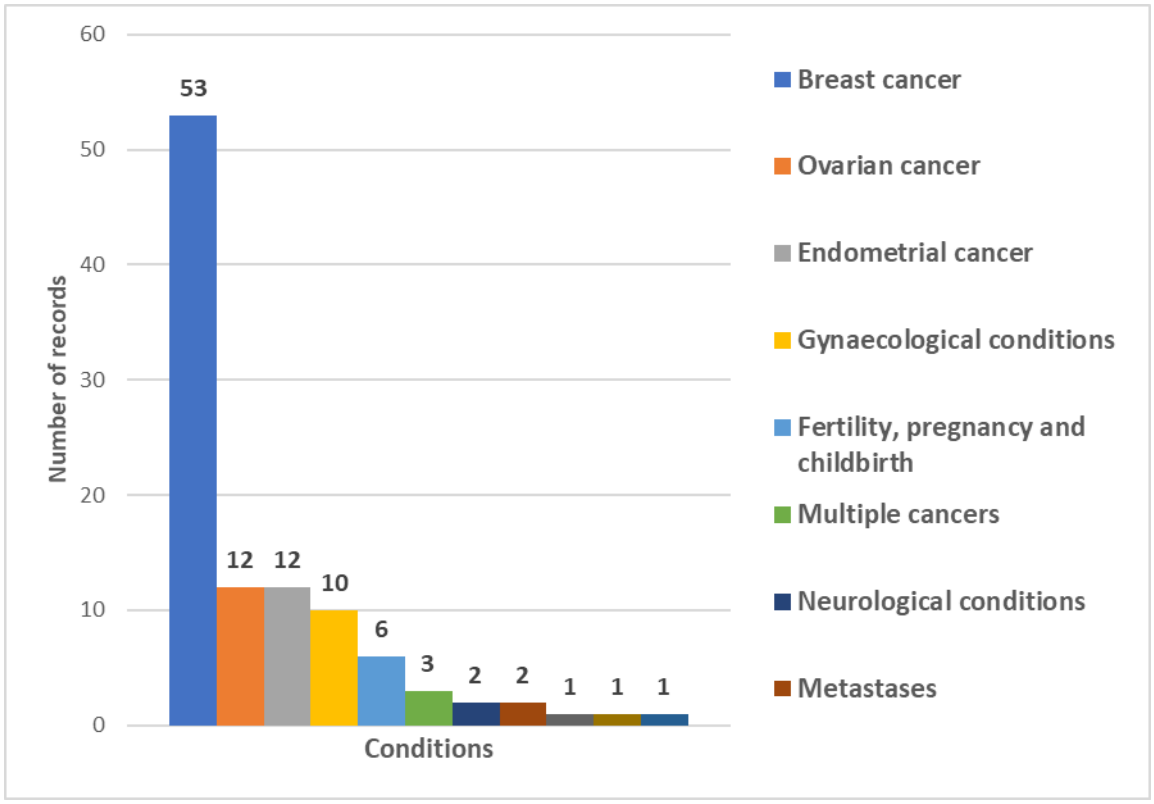

Figure 3. Number of records with indications affecting the female population classified by NICE condition categories[2]

iii. Indications for paediatric population

303 (16% of total) records were created and updated on MInD for use in the paediatric population (below 18 years) in 2024. These records included other age categories in some cases, while others were mainly for the paediatric population. Of these records, 54 (18%) were in development for cancer indications, and 249 (82%) for non-cancer indications classified by NICE condition categories.

Of the 54 cancer records, blood and bone marrow cancer indications (n=15, 28%) were the most common. This was followed by the multiple cancers category (n=10, 18%), which refers to cases were there were more than one cancer indication on the record, or solid tumours in general, as shown in Figure 4.

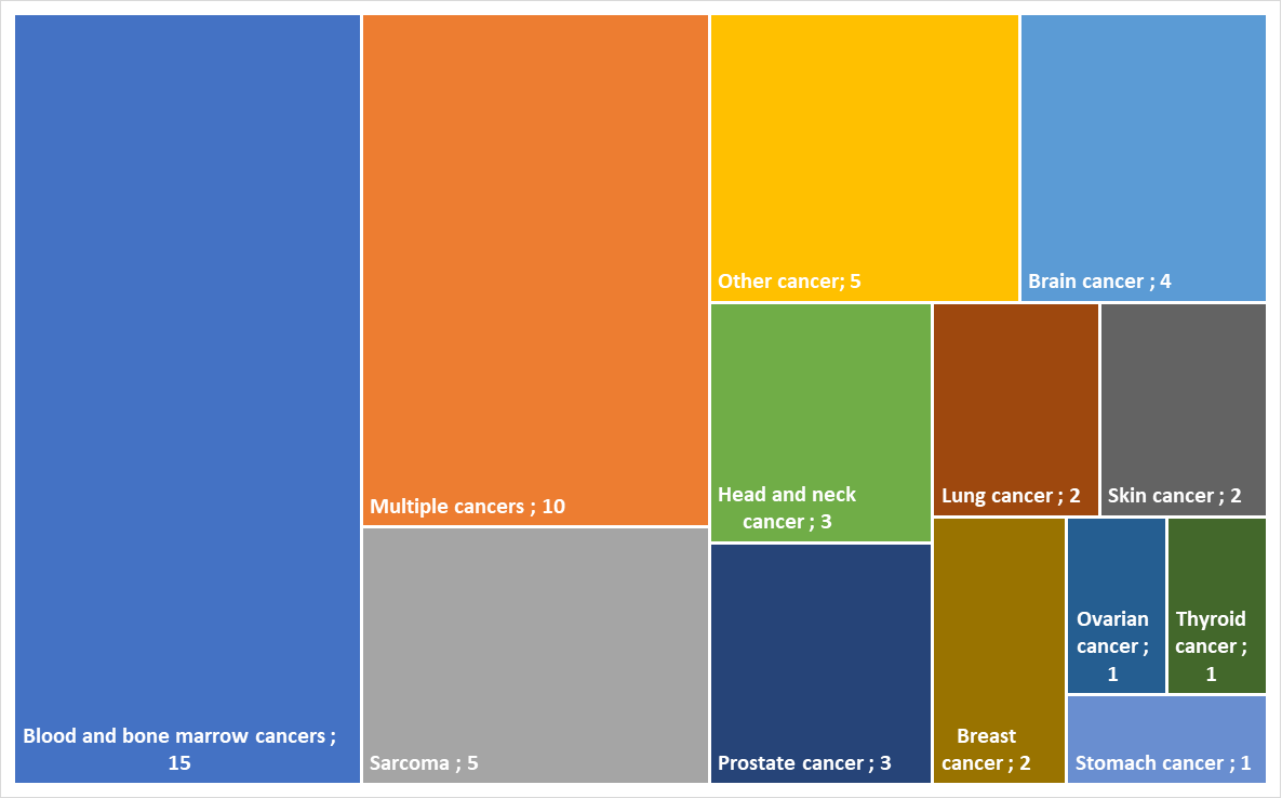

Figure 4. Number of records with cancer indications for the paediatric population classified by NICE condition categories[1]

In the non-cancer category, blood and immune system conditions (n=44, 18%), diabetes and other endocrinal, nutritional and metabolic conditions (n=33, 13%), and neurological conditions (n=26, 10%) were the most frequent, as shown in Figure 5.

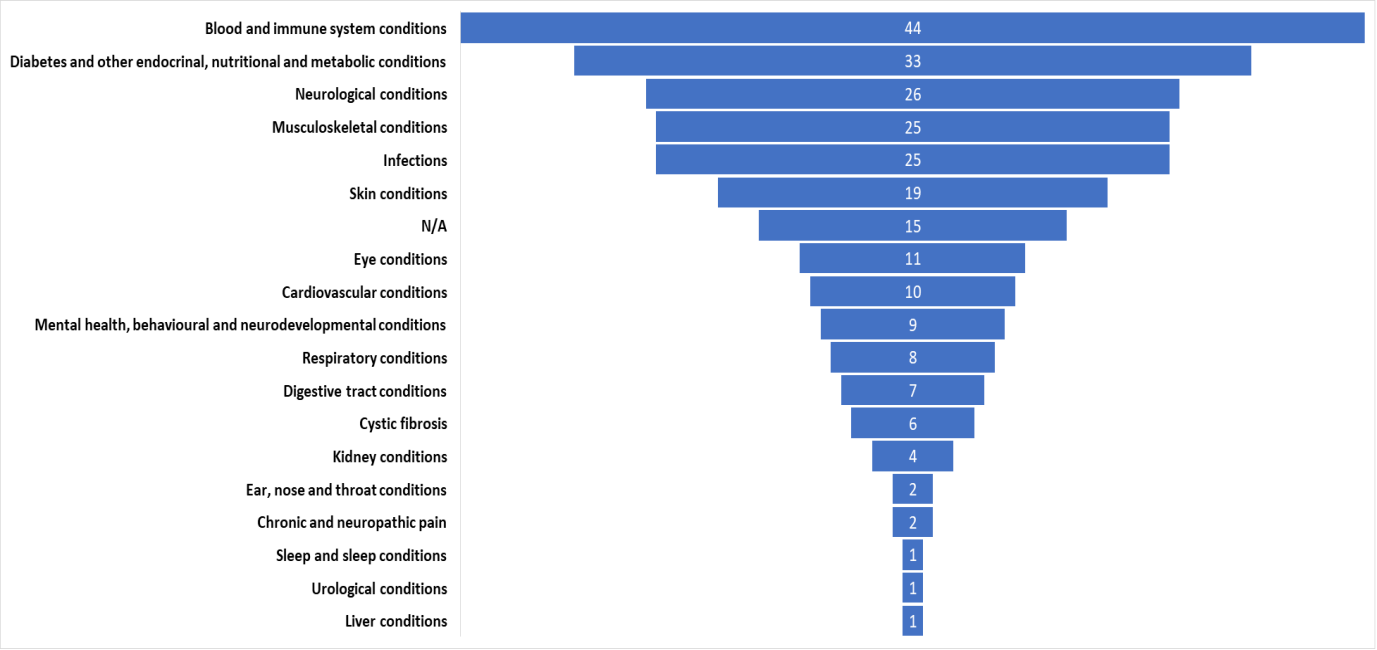

Figure 5. Number of records with non-cancer indications for the paediatric population classified by NICE condition categories[4]

1. Interventions

i. Monotherapies vs Combination therapies

Out of the 1,916 newly created and updated records in 2024, 1,377 (72%) had monotherapy interventions while 539 (28%) had combination therapies. Of the 758 cancer records, there were more combination therapies (n=419, 55%) for cancer indications compared to monotherapies (n=339, 45%). The reverse was the case in non-cancer categories where monotherapy interventions (n=1,038, 90%) appeared more frequently than combination therapies (n=120, 10%).

[4] The category of N/A includes conditions that do not fit well into the other indication categories e.g. anaesthesia

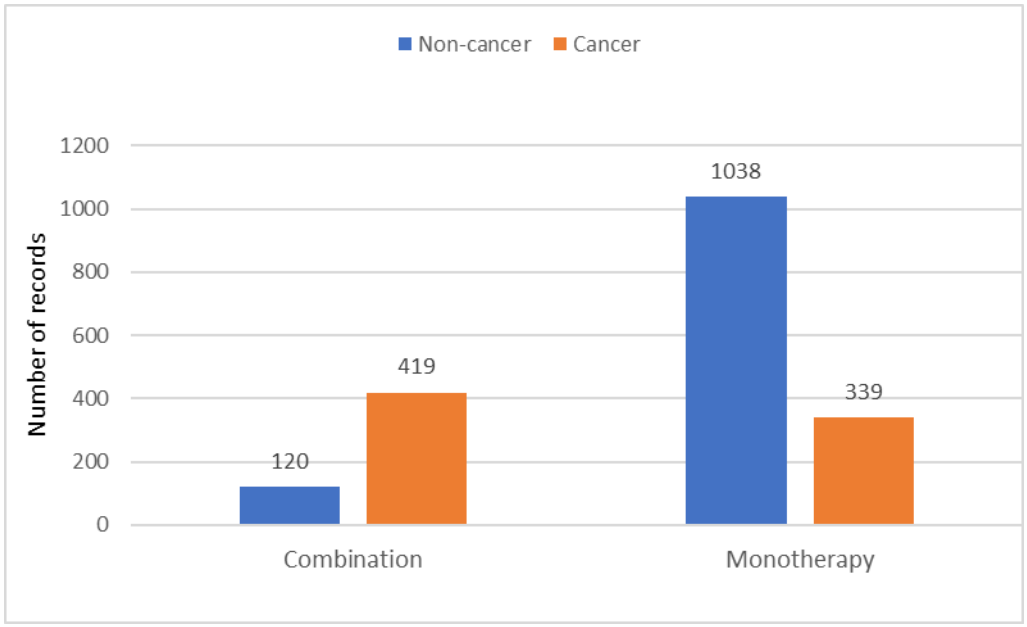

Figure 6. Number of combination therapy and monotherapy records classified by NICE cancer or non-cancer condition categories

ii. 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.[4] Of the 1,916 newly created and updated records in 2024, 87 (5%) records had ATMP interventions. Out of the 87 records, 32 (37%) records had ATMP interventions in development for cancer related indications, while 55 (63%) records had ATMP interventions in development for non-cancer conditions. In the cancer category, blood and bone marrow cancers had the highest number of ATMPs in development (n=22, 69%). This NICE condition category includes indications like multiple myeloma, lymphomas and leukaemia. In the non-cancer category, blood and immune system conditions (n=18, 33%), neurological conditions (n=11, 20%), and musculoskeletal conditions had the highest number of ATMPs in development, as shown in Figure 7. An analysis of the ATMP types showed that there were

52 (60%) records with cell therapies, 33 (38%) records with gene therapy ATMPs, and that two (2%) are tissue-engineered therapies (Figure 8).

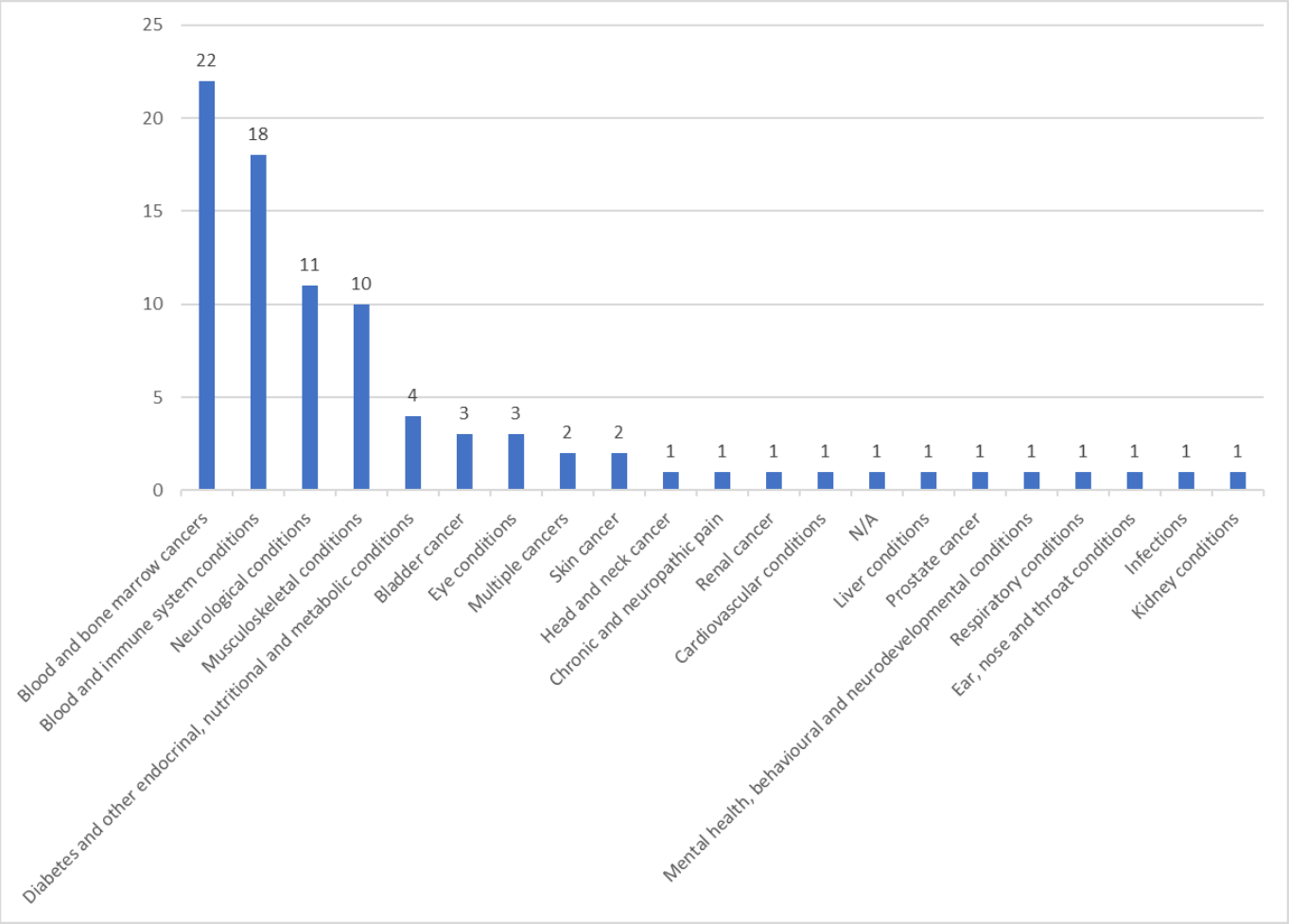

Figure 7. Number of records with at least one ATMP intervention by NICE condition category[5]

[5] The category of N/A includes conditions that do not fit well into the other indication categories e.g. anaesthesia

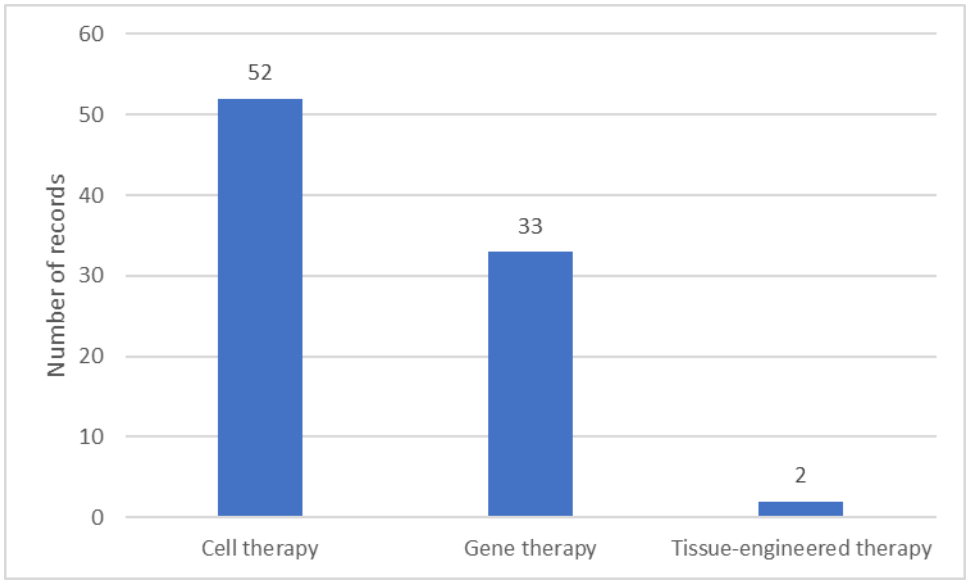

Figure 8. Number of records classified by ATMP type

iii. Biosimilars

The NHS definition of a biosimilar is a medicine which has been shown not to have any clinically meaningful differences from the originator medicine in terms of quality, safety and efficacy.[5] Of the 1,916 newly created and updated records in 2024, 12 (0.6%) records had biosimilar interventions. Out of the 12 records, 3 (25%) records had biosimilar interventions in development for cancer related indications, while 9 (75%) records had biosimilar interventions in development for non-cancer conditions. In the cancer category, lung cancer indications had the highest number of records with biosimilars in development (n=2, 67%). Biosimilar interventions in development for skin conditions appeared most frequently in the non-cancer records (n=4, 44%) out of the 9 newly added and updated records in this category. Denosumab (n=4, 33%) and Ustekinumab (n=4, 33%) were found to be the most

common reference products in the 12 biosimilar records we added and updated on MInD in 2024, as shown in Figure 9.

Figure 9. Number of records with biosimilar interventions by NICE condition category[6]

2. Regulatory information

i. Orphan drugs

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

- It must be intended for the treatment, prevention or diagnosis of a disease that is life-threatening or chronically debilitating;

[6] Within the figure we have included an 'other cancer' category which represents cancer records that that did not fall into any NICE category

- 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 2024, we added and updated 79 records with at least one intervention that had an EMA orphan designation (4% of the total records). Eighteen (23%) of the records had interventions in development for cancer indications, but most records had non-cancer indications (n=61, 77%). Blood and bone marrow cancers were the most common indications seen in the cancer category (n=10, 55%). In the non-cancer category, diabetes and other endocrinal, nutritional and metabolic conditions were the most occurring indications (n=12, 20%), as shown in Figure 10.

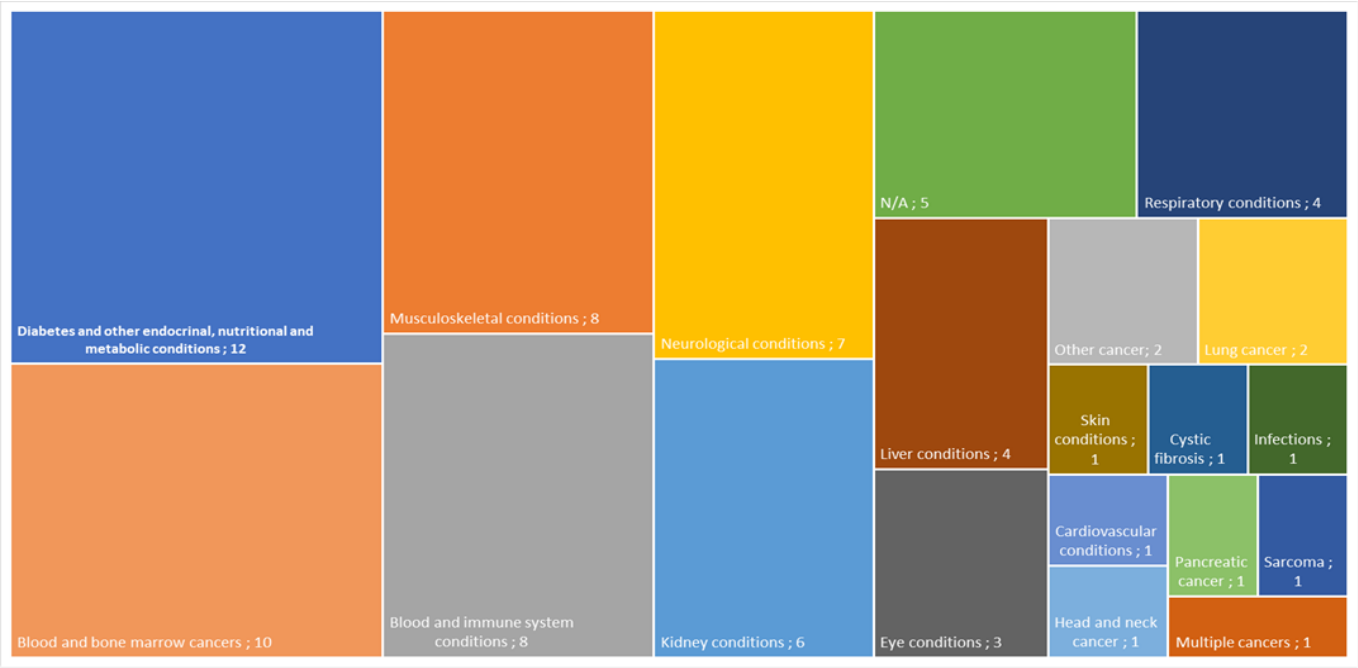

Figure 10. Number of cancer and non-cancer records with at least one orphan drug by NICE condition category[7]

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

[7] The category of N/A includes conditions that do not fit well into the other indication categories e.g. anaesthesia

existing treatments.[7] In 2024, we added and updated 28 records with at least one intervention that had a PRIME designation (1.5% of the total records). 6 (21%) of the records had interventions in development for cancer indications, while most of the other records had non-cancer indications (n=22, 79%). Blood and bone marrow cancers were the most common indications in the cancer category that had at least one PRIME designated drug in development (n=3, 50%). In the non-cancer category, diabetes and other endocrinal, nutritional and metabolic conditions were the most occurring indications (n=6, 27%), as shown in Figure 11.

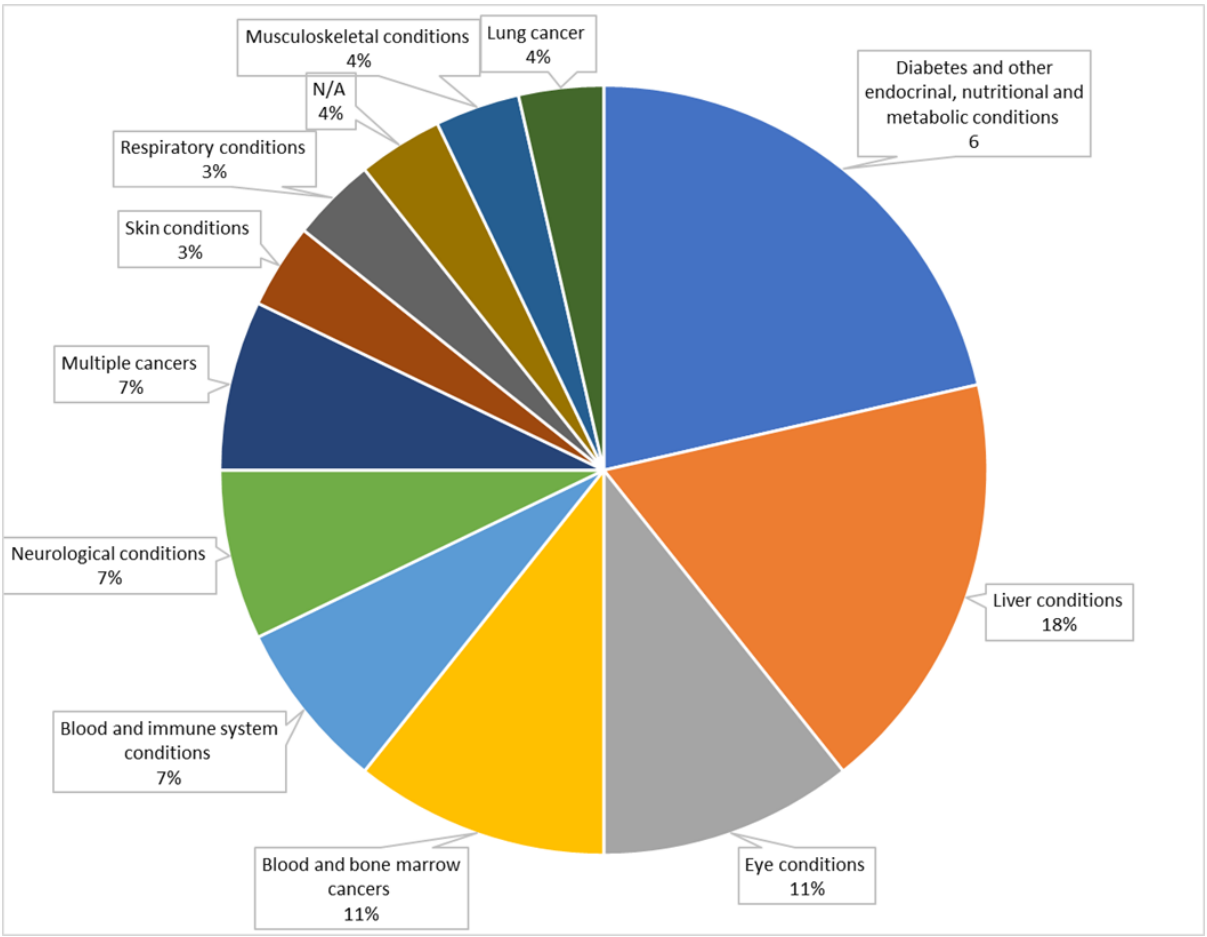

Figure 11. Number of cancer and non-cancer records with at least one PRIME designated drug by NICE condition category[8]

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

[8] The category of N/A includes conditions that do not fit well into the other indication categories e.g. anaesthesia

address an unmet medical need.[8] We added only 2 records (0.1% of the total records) in 2024 with interventions that have been assigned a PIM designation by the Medicines and Healthcare products Regulatory Agency (MHRA). One record was for blood and immune system conditions, while the indication in the other record was 'other cancer' (uveal melanoma) as shown in Figure 12.

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.[9] In 2024, we added and updated 4 records (0.2% of the total records) with an ILAP designation. Two (50%) of these records had diabetes and other endocrinal, nutritional and metabolic conditions as their indications, while the other 2 (50%) were for mental health, behavioural and neurodevelopmental conditions, and multiple cancers respectively as shown in Figure 12.

Figure 12. Records with MHRA ILAP and PIM designations by NICE condition category

3. Sponsors

We identified 788 unique companies that were either the sponsors, collaborators or developers of the clinical trials that were newly created and added on MInD in 2024. From the 1,916 newly added or updated records in 2024, the companies with the highest number of records were Merck Sharp & Dohme Ltd (n=71, 4%), AstraZeneca Ltd (n=66, 3%) and Bristol-Myers Squibb pharmaceuticals Ltd (n=53, 3%).

Of the 758 newly created and updated cancer records in 2024, Merck Sharp & Dohme Ltd had the highest number (n=57, 7.5%), this was followed by Bristol-Myers Squibb Pharmaceuticals Ltd (n=39, 5.1%) and AstraZeneca (n=36, 4.7%). In the 1,158 non-cancer category, Eli Lilly and Company Ltd and AstraZeneca Ltd had an equal and the highest number of records (n=30, 2.6%), this was followed by Sanofi and Novartis Pharmaceuticals UK Ltd with 28 records each (2.4%). We also identified companies that were developing interventions with EMA orphan and PRIME designations. There were 78 records that had at least one orphan or PRIME designated drug., and Pfizer Ltd UK had the highest number of these (n=4, 5%), followed by Intellia Therapeutics and Novartis pharmaceuticals UK Ltd both with 3 records (4%). Of the 6 newly created or updated records in 2024 with MHRA PIM or ILAP designations, Leucid Bio, Rezolute Inc, Lykos Therapeutics, Freeline Therapeutics Ltd, Cambridge Allergy Ltd and Immunocore Ltd were developing these interventions; all had one record each, as shown in Figure 13.

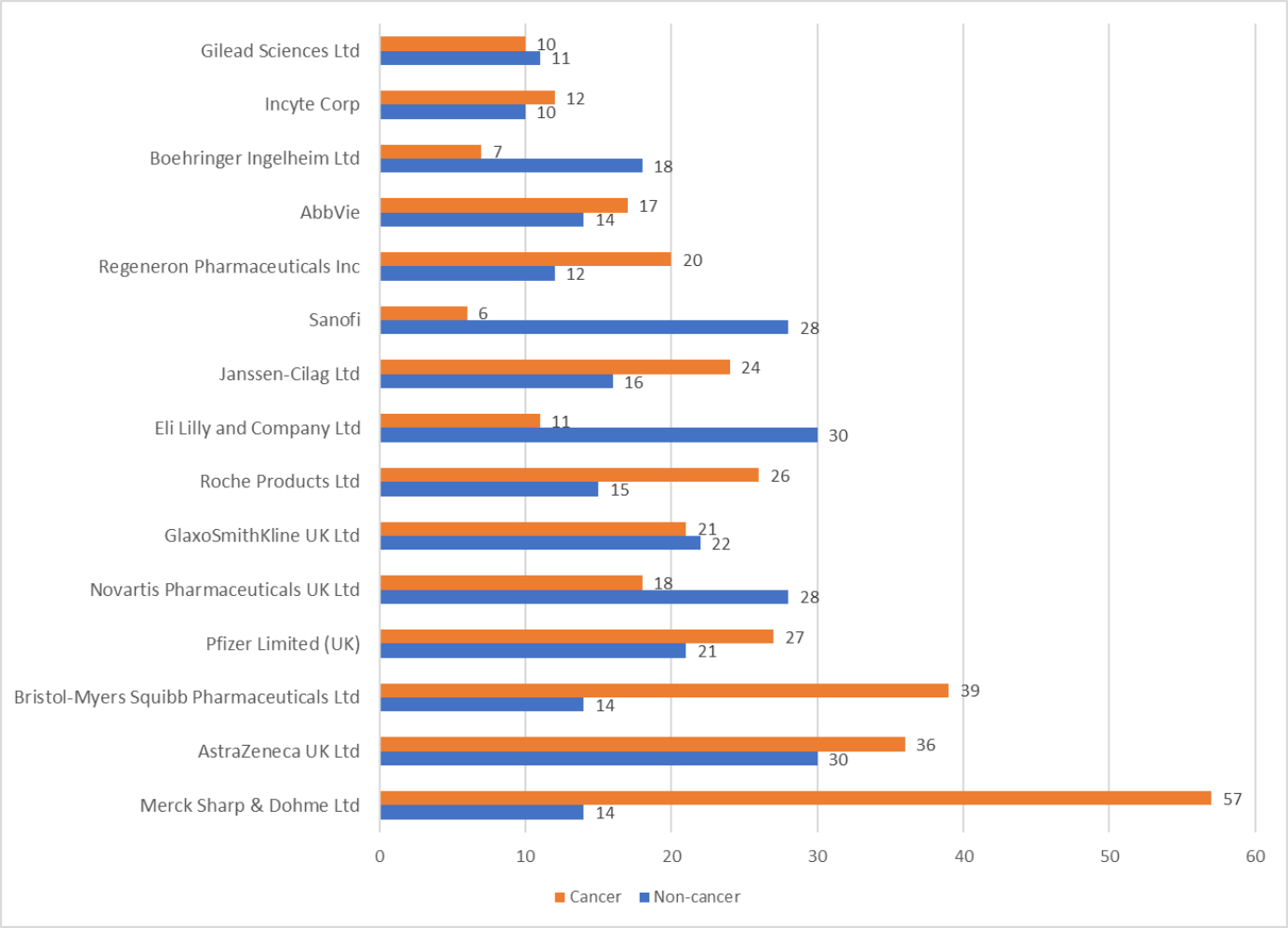

Figure 13. Top 15 companies with the highest number of records by NICE cancer or non-cancer categories.

Conclusion

This report presents a snapshot of the newly added and updated records on IO's MInD database in 2024. We analysed the technology records base on indications, interventions, regulatory information, and sponsor information. We created 1,619 new records and updated 297 with clinical trials in 2024, giving a total of 1,916. 40% of these records were for cancer indications and 60% for non-cancer. 90% of the total records were found to be for monotherapy interventions with the rest being combination therapies. We also identified the total number of interventions in development for use in only the female population, these records represented 5.3% of the total records with breast cancer being the most common indication. In children aged below 18 years, 16% of the total records were identified to be in development for use in this population. Blood and bone marrow cancers, and blood/immune

system conditions were found to be the most frequent indications among the newly created and updated records in 2024 including this population.

When analysing the total number of designations or awards for interventions added or updated in 2024, we identified 79 records with at least one EMA orphan drug, 28 records with at least one PRIME designation drug, 2 records with interventions that had an MHRA PIM designation, and 4 records that had interventions with ILAP designations. Merck Sharp & Dohme Ltd was the commercial sponsor that had the most records both overall, and in cancer indications for 2024. For non-cancer indications, Eli Lilly and company Ltd and AstraZeneca Ltd had the highest number of newly created or updated records in 2024.

Future work

This report is the fifth annual report produced by IO. We aim to produce reports every year to present a snapshot 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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