Medicines Horizon Scanning Annual Report 2023

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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 of 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 2023. It provides: a summary of the horizon scanning strategy devised to identify newly created or updated[1] records within 2023; 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)
- 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, USA, Canada, Australia
- Therapeutic and prophylactic interventions for COVID-19 identified after March 2023 were included as regular technologies, before this date they 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 2023. 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 2023 landscape of medicines in remit for IO

Our analysis showed that from a total of 2,359 records, 1,976 were created and 383 were updated in 2023 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 2,359 records that were created and updated on MInD in 2023, 955 (40%) were for cancer indications and 1,364 (60%) for non-cancer indications. Figure 1 shows the numbers of records for cancer indications as per NICE condition categories. The most common cancerous indication categories were blood and bone marrow cancer (n= 236, 24% of cancer records

[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

added or updated in 2023), multiple cancers (n= 165, 17%), and lung cancer (n= 109, 11%). Figure 2 shows the most common NICE condition categories for non-cancerous indications. The most common non-cancer categories were neurological conditions (n= 161, 12%), infections (n= 149, 11%), and blood and immune system conditions (n= 144, 11%).[3]

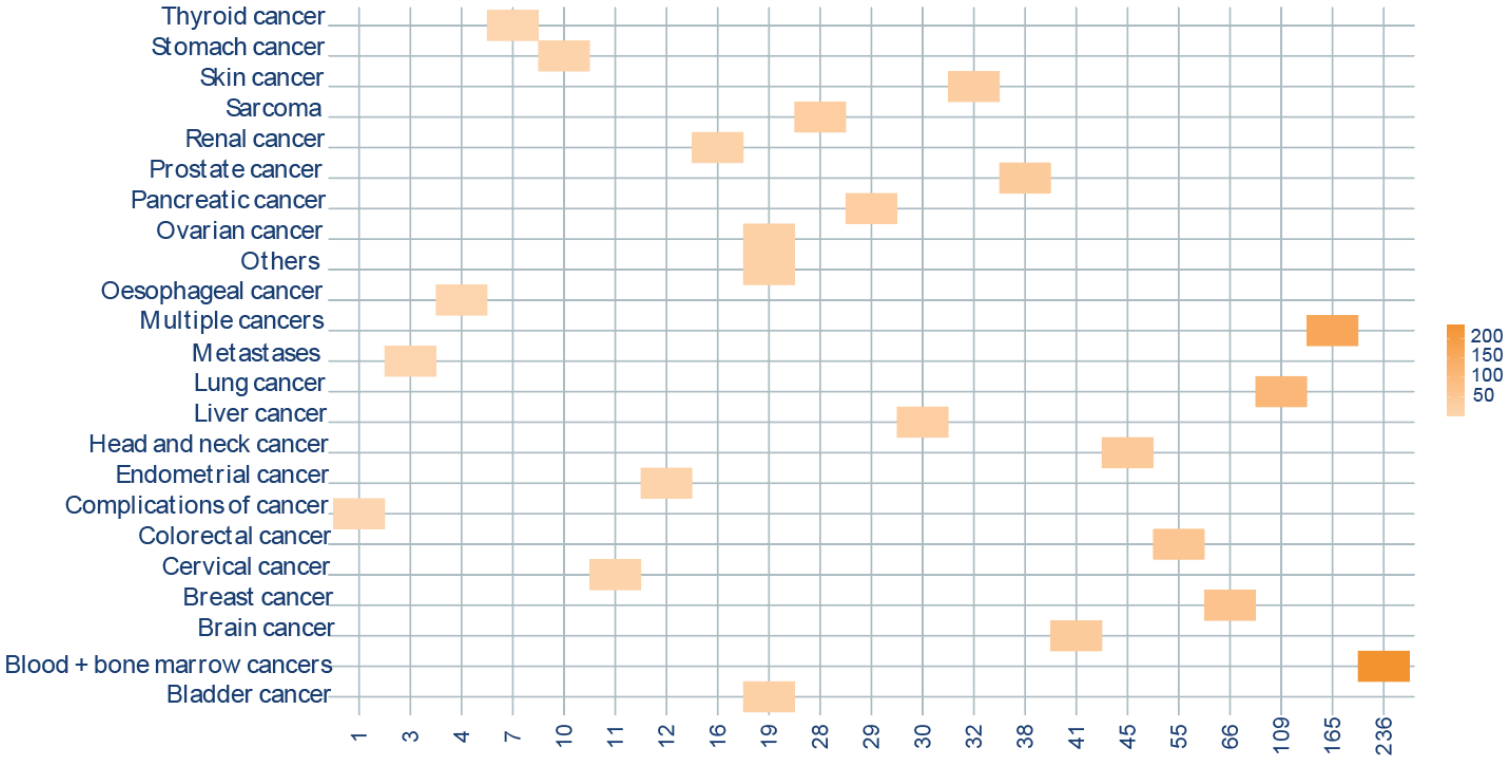

Figure 1. Number of records by NICE cancer condition group [4]

[4] Within the figure we have included an 'Other' category which represents indications that do not fit into a NICE category

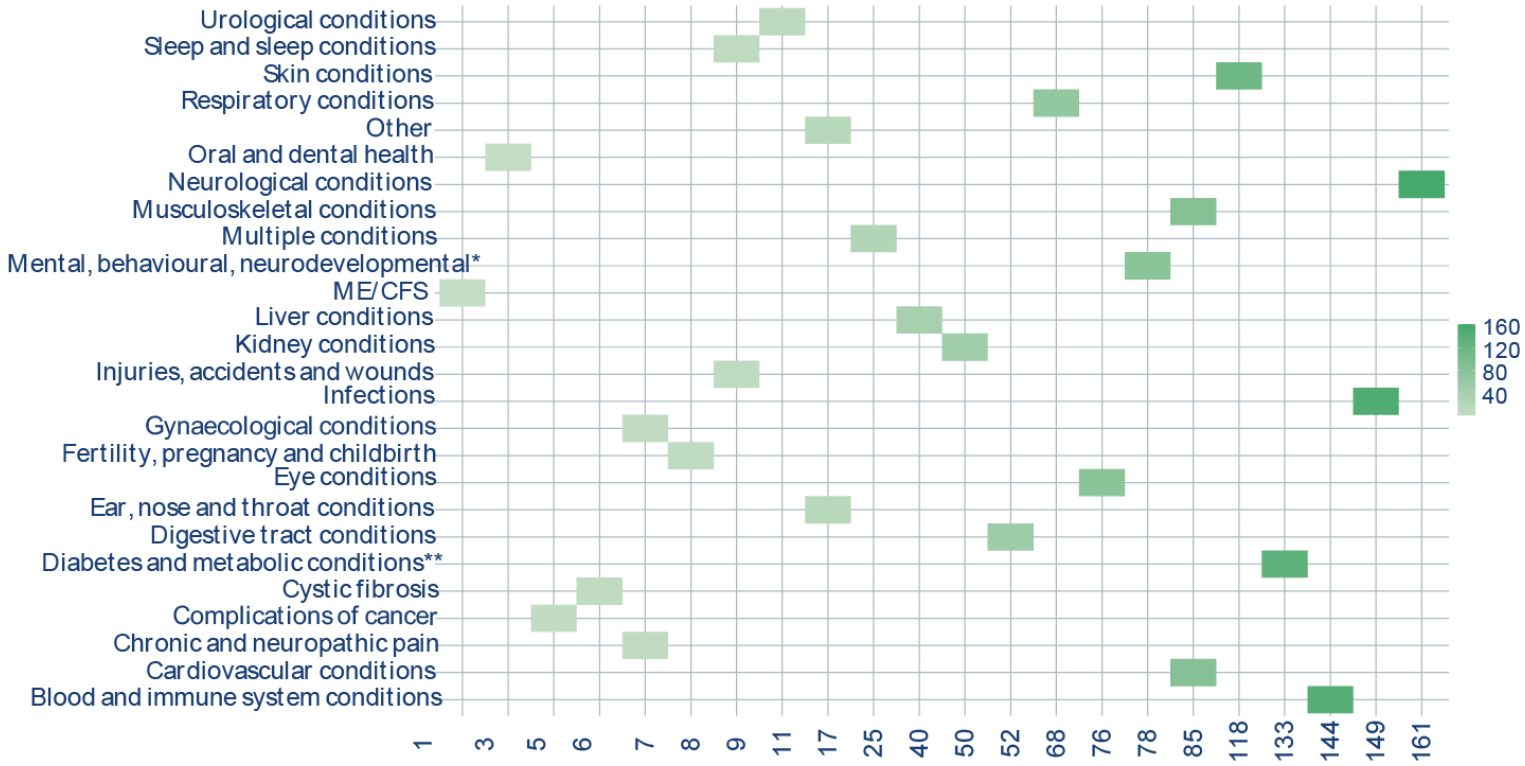

Figure 2. Number of records by NICE non-cancer condition group[5]

[5] *Mental health, behavioural and neurodevelopmental conditions . **Diabetes and other endocrinal, nutritional and metabolic conditions.

ii. Indications for female population group

When looking at records created or updated in 2023 for women's health, we identified 138 (6%) records. Out of these 129 (93%) were for cancer indications with the most common being breast cancer (n= 66, 48%), multiple cancers (n= 21, 15%), and ovarian cancer (n= 18, 13%). Of the non-cancer records identified (n=9, 7%), the most frequently seen were gynaecological conditions (n=3, 2%), followed by skin conditions (n=2, 1%) as shown in figure 3.

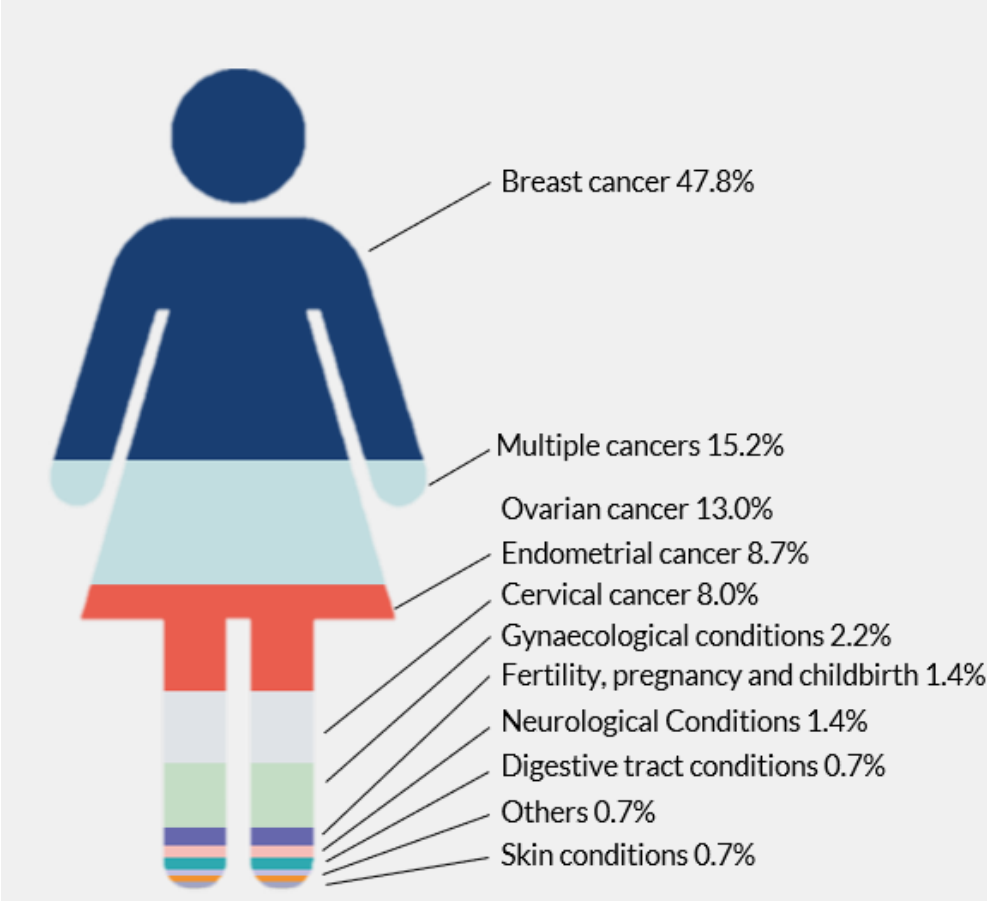

Figure 3. Percentages of all female-only condition records

iii. Indications for paediatric population

In 2023 we added or updated 271 (11%) records that included a paediatric population, either solely or with adults. 47 (17%) of these records were for cancer indications and 224 (83%) for non-cancer indications. The most frequent NICE condition categories for cancer in paediatric populations were blood and bone marrow cancers (n= 14, 30%), multiple cancers (n= 8, 17%), and sarcoma (n=7, 15%) as shown in figure 4a. For non-cancer categories we found that blood and immune system conditions were most common (n= 31, 14%), followed by infections (n= 30, 14%), and diabetes and other endocrinal, nutritional, and metabolic conditions (n= 26, 12%) as seen in figure 4b.

A

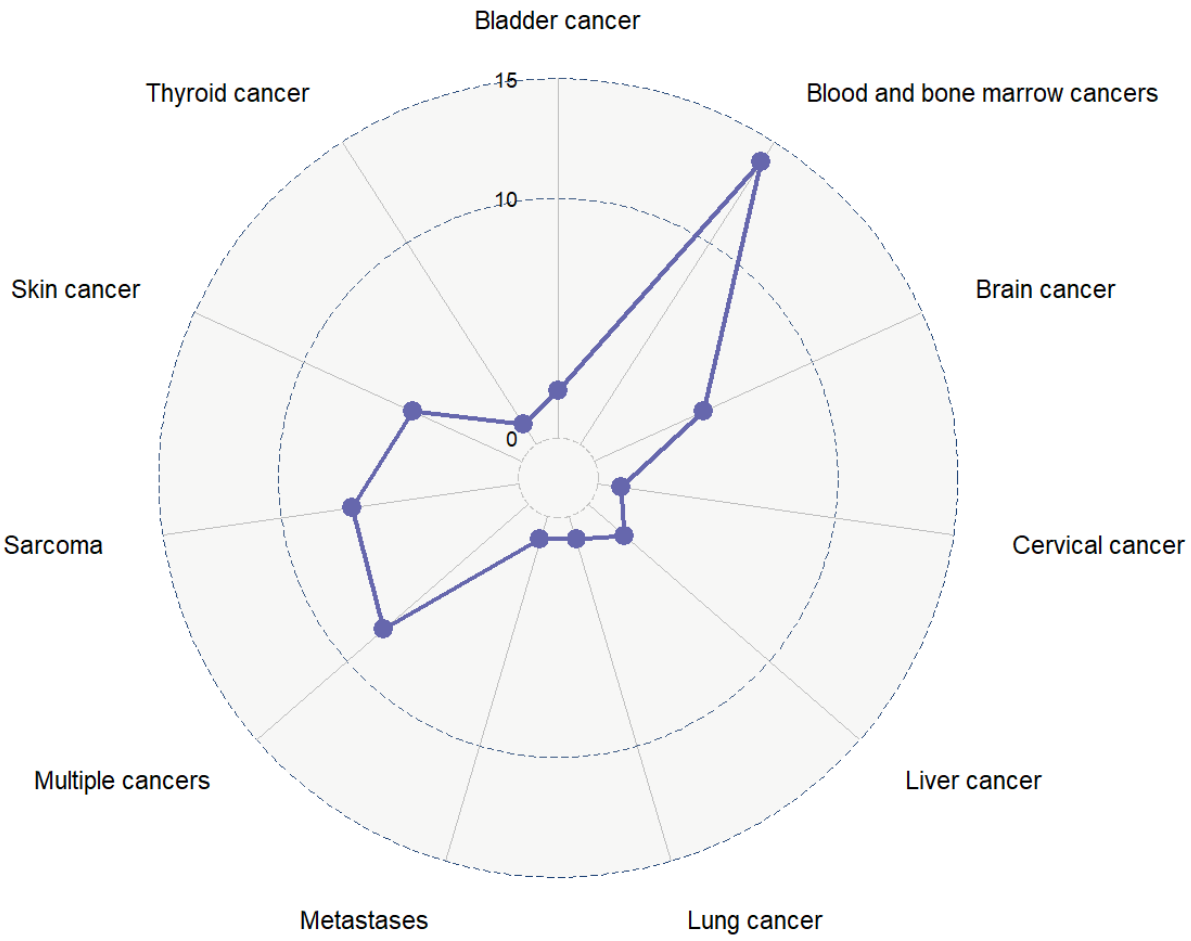
Bladder cancer
Blood and bone marrow cancers
Brain cancer
Cervical cancer
Liver cancer
Lung cancer
Metastases
Multiple cancers
Sarcoma
Skin cancer
Thyroid cancer
15
10
0

Figure 4. Records for paediatric or mixed population by NICE cancer (A) and non-cancer (B) condition group[6]

2. Interventions

i. Monotherapies vs combination therapies

Of the 2,359 added or updated records from 2023 we identified that 1,571 (67%) were for monotherapy technologies, and 788 (33%) were for combination therapies. Within the monotherapy records, non-cancerous indications were most common (n= 1,196, 76%), followed by cancer (n= 375, 24%). The opposite pattern was seen in the combination therapies

[6] The category of N/A includes conditions that don't fit well into the other indication categories e.g. anaesthesia

with cancer indications being more widely studied (n=620, 79%) than non-cancer (n= 168, 21%) (figure 5).

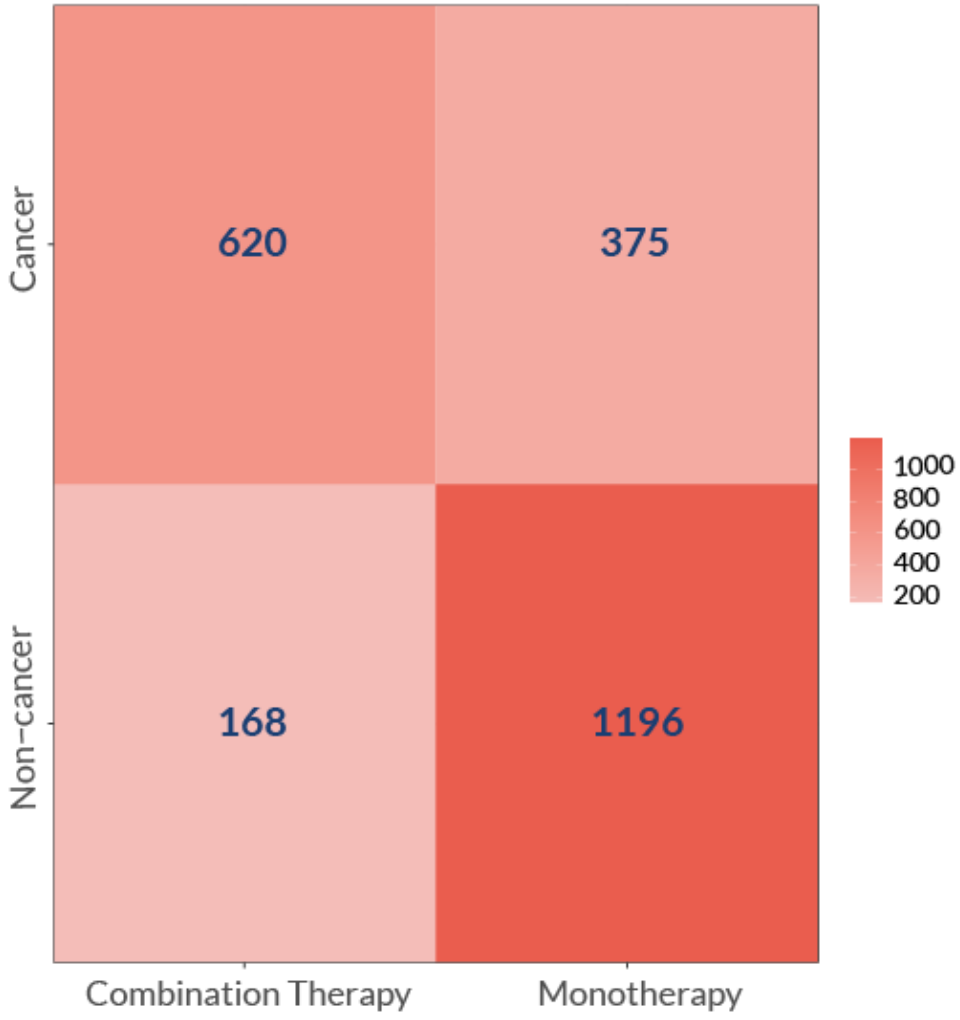

Figure 5. Number of records for monotherapies and combination therapies by type of indication

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] In 2023 we identified 11 ATMP where the record was added or updated representing 0.47% of the total 2023 records. 6 (55%) records were for cancer indications, and all of these fell within the NICE condition category of blood and bone marrow cancers. Within the 5 (45%) non-cancer records we found that musculoskeletal conditions were most common (n= 3, 60%), followed by blood

and immune system conditions (n=1, 20%), and diabetes and other endocrinal, nutritional, and metabolic conditions (n= 1, 20%) (figure 6).

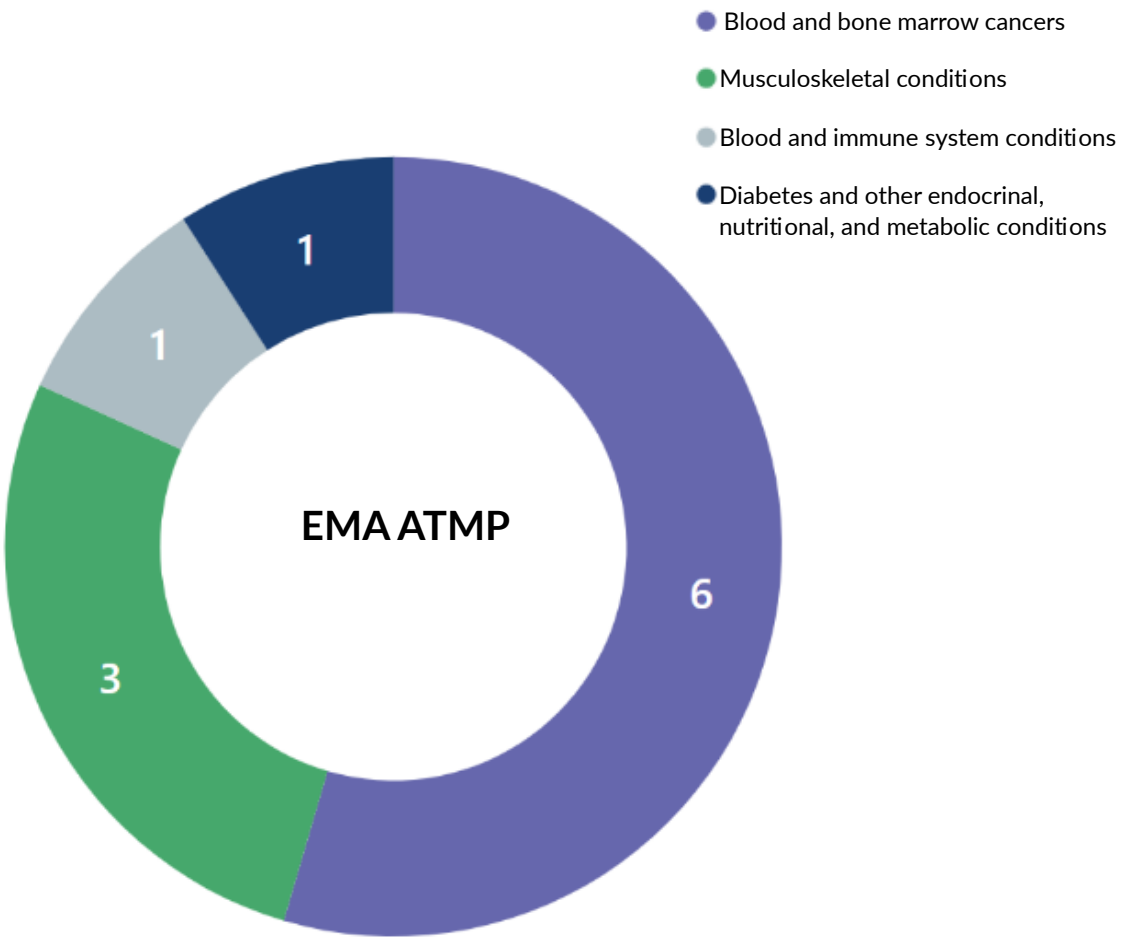

Figure 6. Records containing at least one EMA-classified ATMP by type of condition

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] In the records added/ updated in 2023 only 7 (0.29%) of these concerned a biosimilar. Of these 7, 3 (43%) were for cancer indications, and 4 (57%) were for non-cancer. All of the indications across cancer and non-cancer only had 1 record in each represented NICE condition category,

except skin cancer (n=2, 29%). Each intervention was also only represented in one record each, except for nivolumab biosimilar (n=2, 29%) (figure 7).

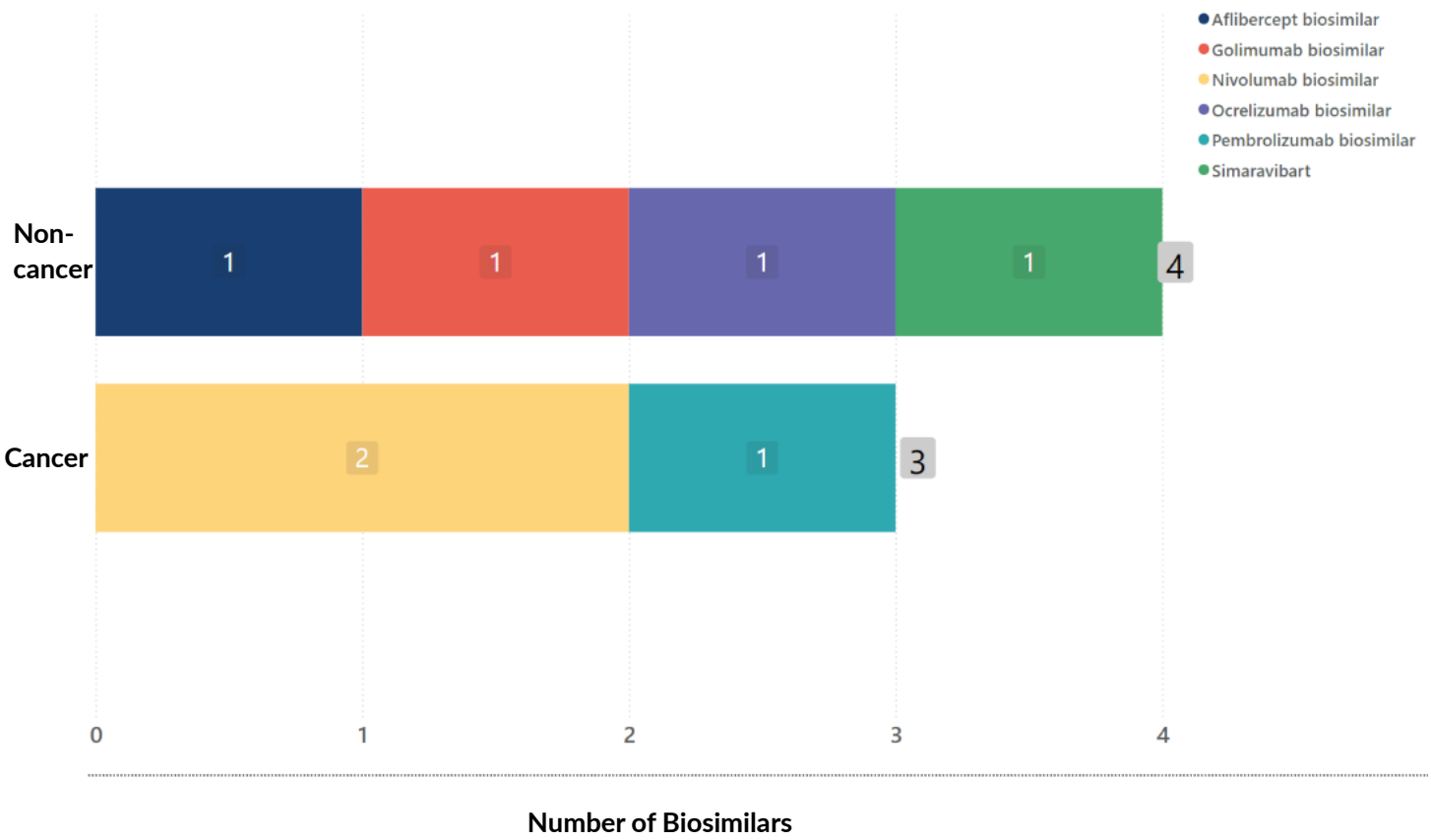

Figure 7. Number of records for biosimilar medicines

3. 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;
- 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.

We added or updated 101 records in 2023 that had an EMA orphan designation (4.3% of the total records) (figure 8). Of these records 32 (32%) were for cancer indications, with blood and

bone marrow cancers most prevalent (n= 18, 18% of orphan records), followed by brain cancer (n= 4, 4%), and liver cancer (n=3, 3%). Non-cancer indications made up 69% of the total (n=69) with blood and immune system conditions most frequent (n= 18, 18%), followed by musculoskeletal conditions (n=10, 10%), and diabetes and other endocrinal, nutritional, and metabolic conditions (n= 8, 8%) (Figure 8).

Figure 8. Number of records and conditions for medicines with orphan drug designation with non-cancer conditions in red and cancer conditions in green

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] We added/ updated 16 records in 2023 that had a PRIME designation, 0.67% of the total number sifted. Of these 16 records, 3 (19%) were for cancer indications with skin cancer, blood and bone marrow cancers, and the 'other' category each having 1 record. Of the non-cancer indications (n= 13, 81%), there were 3 records for each of: neurological conditions; diabetes and other endocrinological, nutritional or metabolic conditions; and blood and immune system conditions. A few technologies were identified that had both EMA orphan and PriME designations (Figure 9).

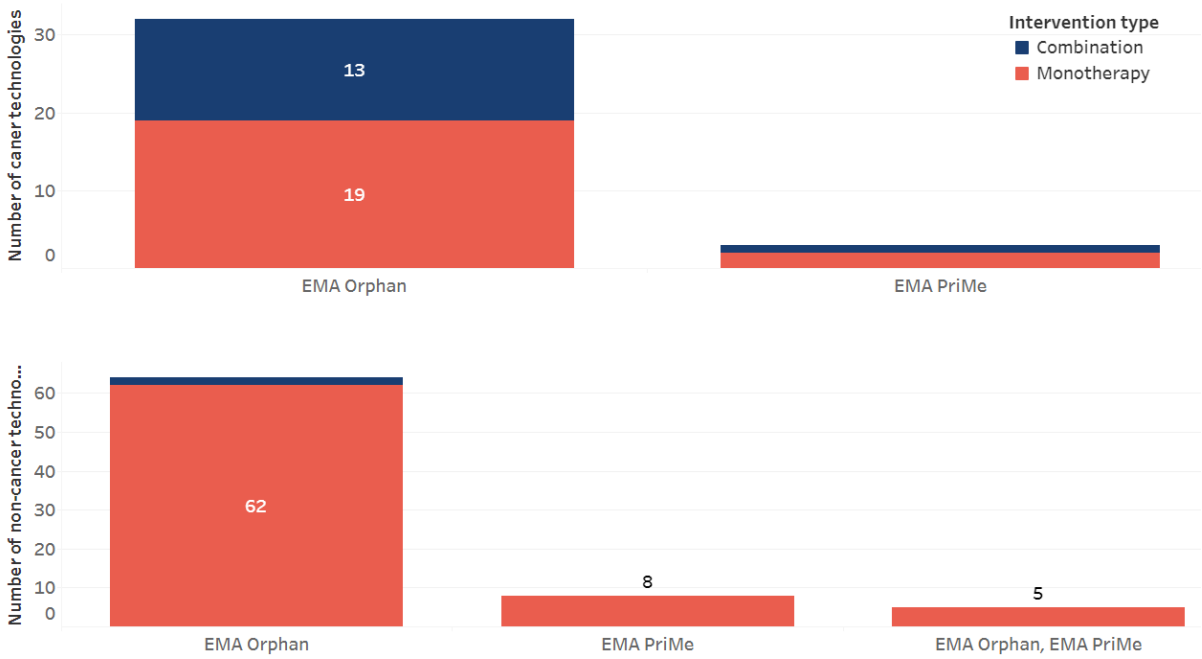

Figure 9. Types of technologies in records with orphan and PRIME designations

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] From the 2,359 records added or updated in 2023 we identified only 2 records with a PIM designation (0.08%). Each of these records were for non-cancer indications and fell under the NICE categories of musculoskeletal conditions and blood and immune system conditions (Figure 10).

iv. Innovation Licensing and Access Pathway

The Innovative Licensing and Access Pathway (ILAP) aims to accelerate the time to market, facilitating patient access to medicines. It comprises an Innovation Passport designation, a target development profile (TDP) and provides applicants with access to a toolkit to support the design, development and approvals process. To quality for ILAP the condition must be life-threatening or seriously debilitating with a significant patient or public health need, with the potential to offer benefits to patients. It must also fulfil one or more of these criteria:

1. innovative medicine such as an advanced therapy medicinal product (ATMP) or new chemical or biological entity or novel drug device combination.
2. medicines being developed in a clinically significant new indication for an approved medicine.

3. medicines for rare disease and/or other special populations such as neonates and children, elderly and pregnant women.
4. development aligning with the objectives for UK public health priorities such as the Chief Medical Officer, Department of Health and Social Care (DHSC) or Life Sciences Sector Deal (including those in devolved administrations where appropriate).

In 2023 we identified only 1 record with an ILAP designation (0.04%). This record was for mental health, behaviour and neurodevelopmental conditions, more specifically post-traumatic stress disorder (Figure 10).

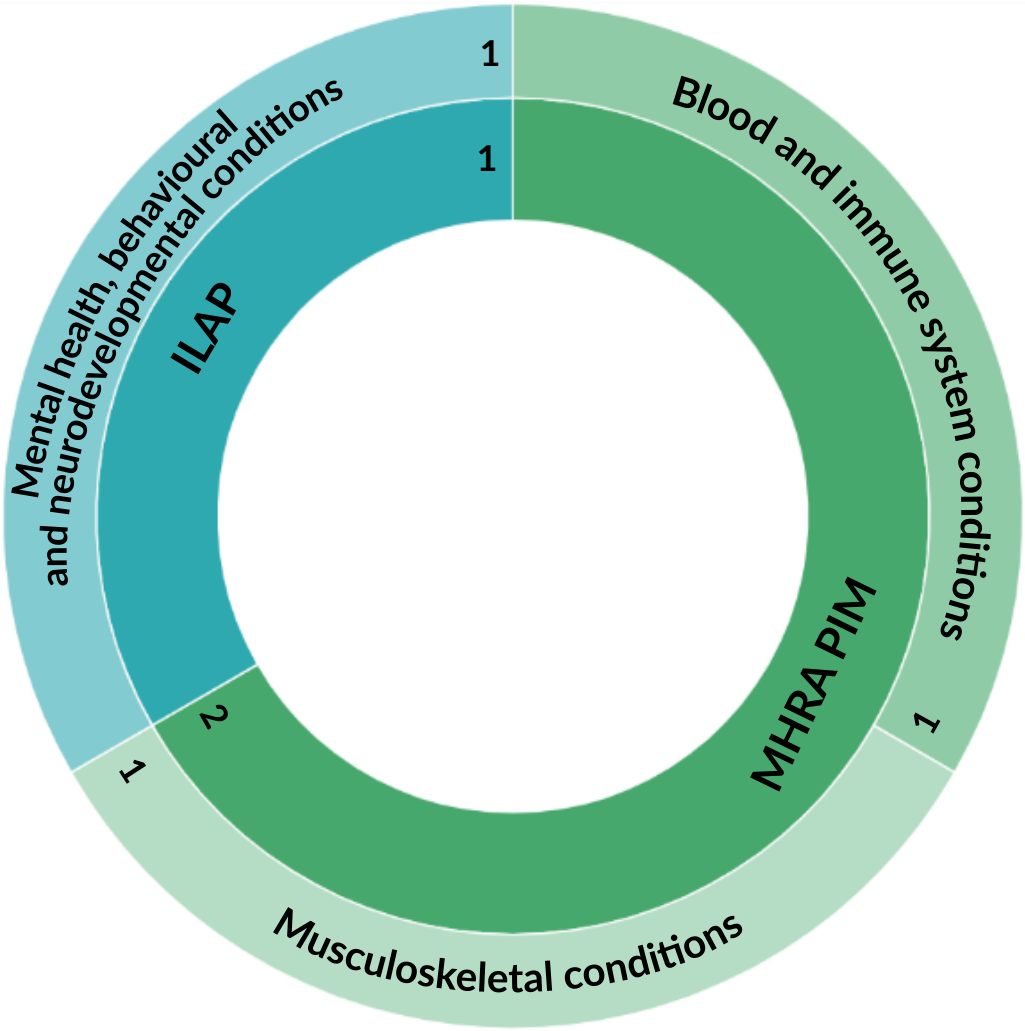

Figure 10. Number of records for MHRA PIM and ILAP awards and the types of conditions

4. Sponsors

When examining the sponsors of the identified records we found that 153 (6.5%) didn't have a company attached to them, this happens when drugs are generic and trials don't have commercial sponsors but records are added to track their development progress for repurposing purposes. Of the records attached to commercial sponsors AstraZeneca UK Ltd had the highest number of records (n= 105, 4.5%), followed by Roche Products Ltd (n= 84, 3.5%), and Merck Sharp & Dohme Ltd (n= 68, 2.8%). When we divided the records added/ updated into cancer and non-cancer we found that AstraZeneca UK Ltd was the most

represented in cancer (n= 73, 3%), followed by Roche Products Ltd (n= 57, 2.4%), and Merck Sharp & Dohme Ltd (n= 48, 2%). In non-cancer indications Novartis Pharmaceuticals UK Ltd had the highest number of records added/ updated in 2023 (n= 39, 1.7%), followed by AstraZeneca UK Ltd (n- 32, 1.4%), and Eli Lilly and Company Ltd (n= 31, 1.3%) (figure 10).

We also examined which companies were developing orphan, PRIME, PIM, ILAP designated drugs. We found that Pfizer Ltd UK had the most records (n= 5, 0.2%), followed by Takeda UK Ltd (n= 4, 0.12%), Vertex Pharmaceuticals Inc (n= 3, 0.12%), Swedish Orphan Biovitrum AB (n= 3, 0.12%), and Merck Sharp Dohme (n= 3, 0.12%). UCB Pharma Ltd, AbbVie, and Lykos Therapeutics each had one technology (0.04%).

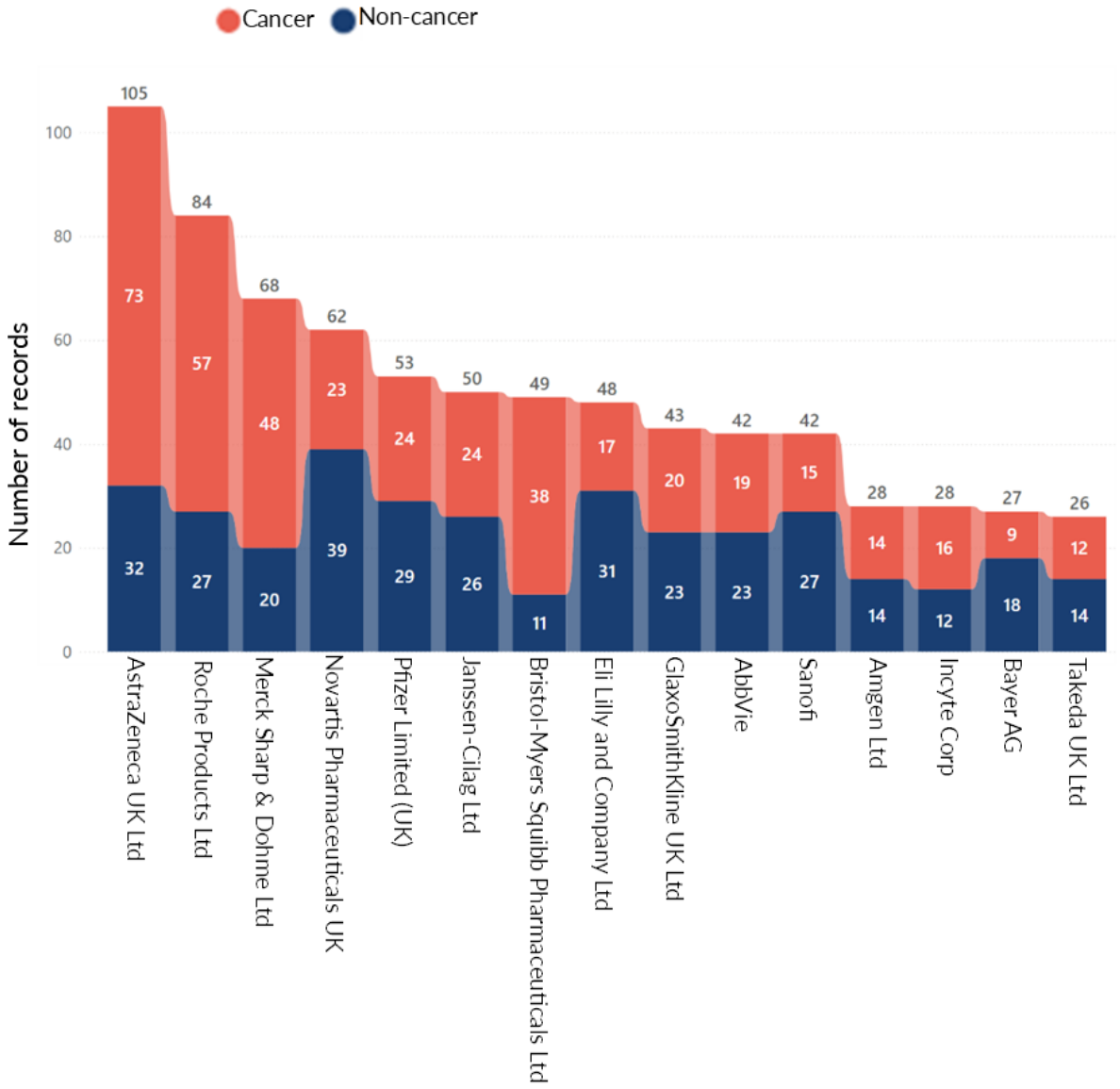

Figure 11. Number of records added/ created in 2023 for each commercial sponsor divided into cancer and non-cancer records

Conclusion

This report presents a snapshot of the new or updated records on IO's MInD database in 2023. We analysed the technology records base on indications, interventions, regulatory information, and sponsor information. We created 1,976 new technology records and updated 383 in 2023, giving a total of 2,359. 40% of these records were for cancer indications and 60% for non-cancer. 67% of the total records were found to be for monotherapy technologies with the rest combination therapies. We also examined the total number of technologies for women's health and found that 6% were specifically in this area in 2023, with breast cancer the most common

indication. When examining the total number of designations or awards for records in 2023 we identified 101 EMA orphan drugs, 16 with a PRIME designation, 2 PIM, and 1 ILAP designation. AstraZeneca UK Ltd was the commercial sponsor that had the most records overall in 2023, and in cancer technologies but for non-cancer indications Novartis Pharmaceuticals UK was most prevalent in records added/ updated in 2023.

Future work

This report is the fourth 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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