Patenting repurposed drugs ("second medical uses")

A common strategy for protecting pharmaceuticals for a new medical use is to initially file a first patent application (e.g., in the UK), to establish a "priority date", then within 1 year (the "priority year"), to file a second application, a PCT (i.e., "international application"), which can later be used to cover countries of interest.

WHAT CAN BE PATENTED

i. A known drug for a **new use** of that drug. The new use may be treatment or prophylaxis.

Example: Drug A for treating breast cancer (where A was already known for treating diabetes, but not breast cancer).

ii. A new **combination** of known drugs provided the combination leads to a new or improved technical effect.

Example: A composition comprising drug A and drug B for treating breast cancer:

(i) where only A was already known to treat breast cancer, but the use in combination with B leads to a new or improved technical effect such as more efficacious treatment or fewer side-effects, or

(ii) where both A and B were already known to treat breast cancer individually, but together they act synergistically to achieve a better than the expected additive effect i.e., administrating them together results in an effect that is greater than the additive value of administering the two individually.

iii. A new **specific formulation** of a known drug provided the formulation achieves an advantageous technical effect. Specific ranges for the amounts of key components are generally required in the claims. The technical effect may relate to an improved treatment/prophylaxis of a disease such as by achieving a better/more desirable release profile or it may be solving/improving a general formulation issue, such as increased stability or it allows for a particular administration route to be possible.

Example: A solid composition comprising 10 to 20 wt% drug A, 50 to 60 wt% excipient B and 1 to 10 wt% excipient C.

iv. A new **crystal, salt, polymorph** form of a drug provided the crystal/salt/polymorph form achieves an advantageous technical effect. Characterising data from established techniques for identifying crystals/salts/polymorphs (e.g., X-ray diffraction data) is required along with data evidence of its improved technical effect, such as improved stability, solubility or bioavailability versus other crystals/salts/polymorphs forms.

Example: A crystal form of X wherein the X-ray diffraction pattern of the X shows at least five characteristic peaks at 2theta values selected from […].

v. A new **specific dosage regimen** of a known drug (including specific administration methods and/or timing of administration), provided it achieves an advantageous technical effect. This may be, for instance, that a specific low dosage range is unexpectedly still effective for treating the known disease, reducing the amount of API administered to a patient. Or that a drug has only been previously known to be effective when administered via a certain route, but a different route

has been found, which is perhaps easier to administer (e.g., orally – often this particular example may tie in with the formulation).

Examples: A pharmaceutical composition comprising 2 to 10 mg of drug A to be administered as a total dose once a day (where drug A was previously known to be effective at doses above 50 mg per day).

Drug A for the treatment of diabetes wherein drug A is administered orally twice a day.

vi. A known drug for treating a **specific patient group**. This may be a sub-group of the patient population for which the drug is already known. The sub-group may be a sub-type of the disease (e.g., triple negative breast cancer) or it may be based on a patient characteristic (e.g., patients deficient in vitamin D). However, there must be a relationship between the patient group and the pharmaceutical effect claimed (i.e., the patient group cannot be arbitrary). Most often, these sub-groups are identified from analysis of data post clinical trials.

Examples: Drug A for treating a subject with triple negative breast cancer (where A was already known for treating 'breast cancer').

Drug A for treating a subject with pneumonia, wherein the patient has low levels of vitamin D.

WHAT AND HOW MUCH DATA TO INCLUDE

Simply stating in the application that the compound is suitable for treating the newly claimed disease(s) is not enough.

Experimental data are required in the priority application to at least make it *plausible* that the drug has the claimed effect. *In vitro* data is typically adequate to establish plausibility, but ideally *in vivo* data (animal model) should be included in the PCT application to ensure the application reaches the data requirements for *inventive step*. Acceptable initial data are also those showing that the drug has an effect on a metabolic mechanism involved in the targeted disease; the connection between the mechanism can be either already known or it can be demonstrated in the application. Importantly, the data must credibly relate to the disease in question.

WHEN TO FILE WHEN RELYING ON CLINICAL TRIAL DATA

As clinical trials are normally conducted for second medical uses, the first patent application should be filed **before** the clinical trial protocol is published (important to preserve novelty), with the details of the clinical trial protocol in the patent application. Ideally, this will be timed so that the clinical trial results are available within the priority year, so they can be included in the PCT application. If not, it can be difficult to have the data admitted and considered by patent offices and may result in the refusal of the application.

A patent application claiming a drug for treating a new patient group may be filed **after** the clinical protocol is published (but still before the clinical trial results are published), provided that the new patient group was not contemplated in the initial clinical trial protocol.