

Memo

IRDiRC Therapy Counter

June 22, 2016

Objective

One of IRDiRC objectives is to contribute to the development of 200 new rare disease therapies by 2020. IRDiRC monitors the progress towards this goal through the IRDiRC Therapy Counter, whose methodology was previously established by the Sci Sec, and approved by the CA.

Conditions

The IRDiRC Therapy Counter counts new indications of orphan medical products, for which an orphan medicinal product is defined as a therapy with an orphan designation and a rare disease market authorization. The number of new indications is calculated from the information available on EMA and FDA websites. An indication is counted:

- ▶ No prior indication or marketing authorization exists for this product
- ▶ A marketing authorization exists for a different disease to a previous authorization

A new medical product is not counted if:

- ▶ Marketing authorization exist for the same indication as the latest
- ▶ The new marketing authorization is a label extension or a label change
- ▶ A new FDA indication is preceded by a prior EMA marketing authorization, and vice versa

Any medicinal product losing orphan designation or marketing approval for an indication will be removed from the count. The counter is updated monthly.

Quality control

Yearly, a full extraction of all orphan drugs in both the EMA and FDA listings is performed, to verify that drugs have not lost their orphan status or have not been removed from the market.

Issues

At present, several issues have arisen with the current drug count:

- ▶ Are “new therapies” the same as “new indications”?
- ▶ As indications are counted, not orphan medicinal products, no information is given about the unique number of new orphan medicinal products
- ▶ No information is provided about the number or rare diseases that have become treatable

Possible action items

- ▶ Clearly define (in text) “new therapies”
- ▶ Make a second counter with “unique orphan medicinal products”, or define them in the text
- ▶ Define number of new, treatable diseases in text