THE PRESCRIRE AWARDS FOR 2025

The annual Prescrire Awards are granted in total independence by the Prescrire Editorial Staff.

2025 Prescrire Drug Awards

Every month, Prescrire's Editorial Staff help health professionals decide which of the multitude of drugs on the market are worth adding to their list of useful treatment options, and which are no better than the standard treatment, or indeed worse, and to be avoided. We do this by conducting systematic, critical analyses of the relevant evaluation data available on new drugs, new indications, new pharmaceutical forms and new dose strengths authorised in Europe or in France. European authorisations account for the majority and are the focus of our English edition, *Prescrire International*. We also regularly re-examine the harm-benefit balance of drugs we have previously analysed, when new relevant data come to light.

The 2025 Prescrire Drug Awards were attributed by Prescrire's multidisciplinary team, based on the reviews published in the Marketing Authorisations section of our French edition in 2025, free from the influence of any companies or public agencies involved in the healthcare sector.

5 Prescrire Drug Awards granted in 2025, but no Pilule d'Or

None of the drugs whose evaluation data were analysed by Prescrire in 2025 represented a major therapeutic advance worthy of a Pilule d'Or (Golden Pill Award). Three drugs authorised for rare diseases earned a place on the 2025 Honours List, and two drugs were deemed "Noteworthy".

Cerliponase alfa in neuronal ceroid lipofuscinosis type 2: reduces mortality and slows disability progression. Neuronal ceroid lipofuscinosis type 2 is a rare enzyme deficiency of genetic origin. It presents as progressive cerebral and retinal degeneration, usually between the ages of 2 and 4 years. Most affected children develop multiple disabilities, including profound intellectual disability, and become visually impaired. They generally die in adolescence.

Cerliponase alfa (see also pp. 126-127) is a recombinant form of the deficient enzyme, administered by infusion into a cerebral ventricle. Non-comparative trials suggest that it greatly reduces mortality. In one trial, for example, after a follow-up of about 5 years, none of the patients treated with *cerliponase alfa* had died, versus 24% of the historical controls. In another trial, the estimated 10-year mortality among *cerliponase alfa*-treated patients was 4%, versus 34% in historical controls. It seems to slow disability progression, although considerable disability often remains in the mid to longer term.

Cerliponase alfa's adverse effects are frequent and sometimes serious, and include convulsions, hypersensitivity reactions and complications related to its route of administration, including meningitis.

Brineura® earned a place on the 2025 Honours List because it represents a therapeutic advance in a rare disease, despite the considerable disability that often persists in treated patients, the disadvantages associated with its route of administration, and its adverse effects. These drawbacks must be carefully explained to the child's family, and taken into account when deciding whether or not to initiate this treatment.

Nusinersen and onasemnogene abeparvovec in spinal muscular atrophy before the onset of symptoms: reduced mortality and disability. Spinal muscular atrophy is a rare genetic disorder that leads to progressive neuromuscular degeneration. In its most serious

Pilule d'Or

A Pilule d'Or (Golden Pill) is awarded to drugs that represent a major therapeutic advance in a particularly poorly served field.

None awarded for 2025

2025 Honours List

Drugs included on the Honours List constitute a clear advance for some patients compared with existing therapeutic options, albeit with limitations.

Brineura® (*cerliponase alfa*) - BioMarin

in neuronal ceroid lipofuscinosis type 2 disease (*Prescrire Int* n° 281)

Spinraza® (*nusinersen*) - Biogen Idec

in spinal muscular atrophy before the onset of symptoms (*Prescrire Int* n° 278)

Zolgensma® (*onasemnogene abeparvovec*) - Novartis Gene Therapies

in spinal muscular atrophy before the onset of symptoms (*Prescrire Int* n° 278)

Noteworthy

Drugs deemed "Noteworthy" provide a modest improvement in patient care.

Alecensa® (*alectinib*) - Roche

first-line treatment in inoperable or metastatic ALK-positive non-small cell lung cancer (*Prescrire Int* n° 275)

Sirturo® (*bedaquiline*) - Janssen-Cilag

first-line treatment in pulmonary multidrug-resistant tuberculosis (*Prescrire Int* n° 274)

form, affected infants have severe motor disability, and severe respiratory and feeding difficulties. If left untreated, they often die before the age of 2 years. Less serious forms develop during childhood, and have less impact on motor and respiratory function, and on life expectancy.

Nusinersen is an "antisense" oligonucleotide designed to increase the synthesis of the deficient protein. It is administered intrathecally (into the cerebrospinal fluid) at least 3 times per year. *Onasemnogene abeparvovec* is a gene therapy product administered as a one-time intravenous infusion. Each of these substances was evaluated in one non-comparative clinical trial in a few dozen infants with a genetic diagnosis of spinal muscular atrophy and who had not yet developed symptoms. After a median follow-up of 3 to 5 years, depending on the trial, all the children were still alive and none required permanent respiratory support. With *nusinersen*, most of the children were able to walk independently. With *onasemnogene abeparvovec*, most of the children were able to walk independently (at least 5 steps) by the age of 1.5 or 2 years.

Nusinersen exposes patients to the serious adverse effects of intrathecal injections, including pain, haemorrhage, meningitis and arachnoiditis. *Onasemnogene abeparvovec* carries a risk of sometimes fatal liver injury and thrombotic microangiopathy.

Spinraza° and Zolgensma° earned a place on this year's Honours List because they both represent a notable therapeutic advance for patients with serious forms of this disease. However, as of 2026, uncertainty persists, in particular because it is impossible to know whether all of the children included in the trials would have developed the most severe form of the disease without treatment.

Alectinib as first-line treatment in inoperable or metastatic ALK-positive non-small cell lung cancer: reduced mortality in two trials. Two non-blinded randomised trials compared *alectinib* versus *crizotinib* (both of which are antineoplastic drugs that inhibit various tyrosine kinases, including ALK) in a total of 490 patients with inoperable or metastatic non-small cell lung cancer harbouring a mutation in the *ALK* gene, who had not yet received treatment. After a median follow-up of 2 to 5 years, mortality was about 34% in the *alectinib* groups, versus 41% in the *crizotinib* groups (statistically significant difference).

Alectinib has the adverse effects of ALK inhibitors, in particular: interstitial lung disease, hepatic disorders, QT prolongation, gastrointestinal disorders and visual disturbances. In these two trials, one-quarter to one-third of the patients in each group experienced at least one serious adverse event.

One of the essential principles of experimental science is to demonstrate the reproducibility of the result of a scientific experiment, by conducting at least two experiments. This confirms that the results are not due to chance alone or to a flaw in the experimental design. This principle also applies to the clinical evaluation of drugs. Yet, all too often, antineoplastic drugs are only evaluated in a single randomised comparative trial.

Alecensa° was awarded a place as a Noteworthy drug because it was shown to extend survival in two randomised comparative trials.

Bedaquiline in first-line treatment in pulmonary multidrug-resistant tuberculosis: markedly shortens treatment duration. Tuberculosis is a potentially fatal, contagious, infectious disease that usually affects the lungs. Multidrug-resistant tuberculosis is treated with a combination of antituberculous drugs for several months, or for more than 1.5 years with certain regimens.

The antituberculous drug *bedaquiline* was authorised for use as part of a combination regimen with other antibiotics, as first-line treatment for multidrug-resistant tuberculosis. Its evaluation in this situation is based on several randomised trials conducted in hundreds of patients. Combinations of antituberculous drugs that included *bedaquiline* shortened the duration of treatment by several months compared with the combinations previously recommended by the World Health Organization (WHO), while providing similar bacteriological and clinical efficacy.

Bedaquiline has frequent adverse effects, including QT prolongation and liver injury. It has numerous foreseeable drug interactions, which can persist for several months after treatment cessation, because its elimination half-life is about 5 months.

Sirturo° earned the title of Noteworthy drug, because it markedly shortens the duration of treatment in multidrug-resistant tuberculosis, without compromising efficacy.

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2025 Prescrire Packaging Awards

When Prescrire evaluates a drug's harm-benefit balance, its packaging is an important consideration. Does the packaging help ensure the safety of patients, their families and caregivers? Do any aspects of the packaging increase the risk of medication errors or pose a particular danger? Is the packaging well-designed from the users' perspective, enabling accurate measurement of the doses to be administered, for example?

Our rigorous analysis of a drug's packaging takes many factors into account, including: the clinical situations in which the drug will be used; the patients liable to receive it, such as pregnant women, children, or older adults or patients with a disability who may, for example, have reduced manual dexterity; whether family members, carers or healthcare professionals will prepare and administer the drug; the context in which it will be used (e.g. in a healthcare facility, possibly in an emergency setting); and whether it will be obtained on prescription or on the advice of a community pharmacist.