THE PRESCRIRE AWARDS FOR 2017

The three annual Prescrire Awards, for Drugs, Packaging and Information, are granted in total independence by the Prescrire Editorial Staff. The rules governing the three Prescrire Awards are available online at english.prescrire.org.

2017 Prescrire Drug Awards

New products or new indications eligible for the Prescrire Drug Awards are those evaluated during the previous year in the New Products section of our French edition.

E ach month, the *Prescrire* Editorial Staff publish systematic analyses of the data available on: new drugs, new indications authorised for existing drugs, and existing drugs marketed in a new form or with different packaging. The goal is to help readers distinguish, among the plethora of new products, those worth adding to their list of useful therapies, those worth using instead of older products, and those to be avoided.

Our analyses are based on rigorous procedures, described in detail at english.prescrire.org. The *Prescrire* Editorial Staff conduct these analyses free from any industry or institutional influence. Our independence is made possible by the fact that we are financed exclusively by our subscribers, carry no paid advertising in either the French or the English edition, and receive no grants or subsidies of any kind.

The *Prescrire* Drug Awards are compiled at the end of each year, based on the reviews published that year in our French edition, and taking into account any new data made available since the initial articles were published. These awards honour drugs that constitute a therapeutic advance, in that they offer better efficacy, less frequent or less severe adverse effects (for similar efficacy), or safer or easier administration.

Three drugs received a Prescrire Award for 2017. Three drugs received a Prescrire Drug Award this year: one that earned a place on the Honours List, and two that were deemed "Noteworthy" (see overleaf).

Asfotase alfa (Strensiq[°]) in perinatal and infantile hypophosphatasia. Hypophosphatasia is a rare inherited disease caused by an enzyme deficiency. Perinatal forms have a mortality approaching 100%, while over 50% of babies who develop signs and symptoms before the age of 6 months (infantile forms) die in the first year of life.

Asfotase alfa is a recombinant protein that reproduces the activity of the deficient enzyme. The results of two non-comparative clinical trials in a

"PILULE D'OR" NOT AWARDED FOR 2017



Pilule d'Or / Golden Pill

The Pilule d'Or (Golden Pill) is granted to drugs that constitute a major therapeutic advance in a field in which no treatment was previously available.

2017	NOT AWARDED			
2014 (Prescrire Int n° 157)	ORPHACOL° (cholic acid)			
2007 (Prescrire Int n° 94)	CARBAGLU° (carglumic acid) (a second look)			
2006 (Prescrire Int n° 88)	ORFADIN° (nitisinone)			
1998 (Prescrire Int n° 40)	CRIXIVAN° (indinavir)			
1996 (Prescrire Int n° 28)	DIGIDOT° (<i>digoxin-specific antibody</i>) (a)			
1992 (Prescrire Int n° 4)	SURFEXO° (pulmonary surfactant) (a)			
1989 (Rev Prescrire n° 92)	EPREX° (epoetin alfa) • MECTIZAN° (ivermectin)			
1988 (Rev Prescrire nº 81)	LARIAM° (mefloquine) • RETROVIR° (zidovudine)			
1987 (Rev Prescrire nº 71)	LUTRELEF° (gonadorelin) • DÉCAPEPTYL° (triptorelin)			
1986 (Rev Prescrire nº 61)	ZOVIRAX [®] IV and tablets (aciclovir)			
1983 (Rev Prescrire nº 31)	LOPRIL° (<i>captopril</i>)			
1981 (Rev Prescrire n° 10)	VACCIN HEVAC B° (hepatitis B vaccine) (a)			
No Golden Pill was awarded for 1982, 1984, 1985, 1990, 1991, 1993–1995, 1997, 1999–2005, 2008–2013, 2015, or 2016.				

a- No longer marketed in France as of 2017.

Honours List

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

 STRENSIQ° (asfotase alfa) Alexion
 Perinatal and infantile forms of hypophosphatasia (Prescrire Int n° 187)

2015 (Prescrire Int n° 162)	Hemangiol [®] (<i>propranolol</i> oral solution)
	 Glivec° (<i>imatinib</i>) Malacef° (intravenous <i>artesunate</i>) Sovaldi° (<i>sofosbuvir</i>)
2010 (Prescrire Int n° 114)	• Glivec° (<i>imatinib</i>)
•	• Glivec° (<i>imatinib</i>) • Herceptin° (<i>trastuzumab</i>)
2006 (Prescrire Int n° 84)	• Egaten° (<i>triclabendazole</i>)
2005 (Prescrire Int n° 77)	• Varivax° (varicella-zoster vaccine)
2004 (Prescrire Int n° 76)	 Diacomit^o (<i>stiripentol</i>) Fuzeon^o (<i>enfuvirtide</i>) Morphine Aguettant^o syrup (<i>morphine</i> oral solution) (a)
2003 (Prescrire Int nº 66,69,74)	• Carbaglu° (carglumic acid) • IVheBex° (hepatitis B immunoglobulin) • Meningitec° (conjugate meningococcal C vaccine)(a

Drugs were included on the Honours List every year between 1981 and 2007. No drugs were included for 2008, 2009, 2011–2013, or 2016. The full list of drugs included on the Honours List from 1981 to 2013 can be found in *Prescrire Int* n° 147 p. 79.

a- No longer marketed in France as of 2017.

total of 70 infants and children younger than 5 years old, all treated with *asfotase alfa*, suggest that this drug greatly reduces mortality and the bone disorders associated with this disease. None of the documents identified in our literature search give a breakdown of the results by age group, in particular for infants younger than 6 months old, who have the most serious forms of the disease. This is an important weakness in the drug's evaluation. In addition, the effects of *asfotase alfa* on the complications of the disease other than bone disorders are unknown. The main known adverse effects are injection site reactions, hypersensitivity reactions, and probably ectopic calcifications.

Pertuzumab (Perjeta°) in certain patients with metastatic breast cancer. In women with HER2overexpressing metastatic breast cancer, longerterm results from a single clinical trial in 808 patients showed that adding the anti-HER2 antibody *pertuzumab* to the *trastuzumab* + *docetaxel* combination increases the proportion of women alive after 4 years by about 12 percentage points (54% versus 42%) and prolongs median survival by about 16 months. This benefit was observed in patients who for the most part had not previously received *trastuzumab* and exhibited no cardiac

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

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0017	• PERJETA° (<i>pertuzumab</i>) Roche Metastatic breast cancer, in combi- nation with trastuzumab and doce- taxel in certain patients (Prescrire Int n° 184)					
2017	• TRUVADA° (<i>emtricitabine</i> + <i>teno-fovir disoproxil</i>) Gilead Sciences Prevention of HIV transmission in patients at high risk (Prescrire Int n° 187) (a)					

a- Generic versions with better packaging than Truvada^o were available in France in 2017 (see the Packaging Awards on p. 81).

dysfunction before inclusion in the trial. It is for these women particularly that *pertuzumab* constitutes a therapeutic advance. *Pertuzumab*'s main adverse effects are potentially severe diarrhoea, febrile neutropenia, mucocutaneous disorders, infusion reactions, and heart failure.

Emtricitabine + tenofovir disoproxil (Truvada^o or other brands) to prevent HIV transmission in patients at high risk. The main methods for preventing HIV transmission between serodiscordant partners who have penetrative sex are systematic condom use and antiretroviral treatment of the infected person. However, some people engage in sexual behaviour that carries a high risk of HIV transmission but do not use condoms.

Two clinical trials conducted in men who have anal sex with men have shown that pre-exposure prophylaxis with the antiretroviral combination *emtricitabine* + *tenofovir disoproxil*, taken every day or only during periods of sexual activity, considerably reduces (without eliminating) the risk of acquiring HIV infection, and has acceptable adverse effects. This type of prophylaxis can increase highrisk behaviour in those who mistakenly believe it affords complete protection and could increase the frequency of other sexually transmitted infections. It is also associated with a poorly documented risk of developing HIV infection that is resistant to the antiretrovirals used for prophylaxis.

In summary. The three drugs that received a Prescrire Drug Award for 2017 are indicated for very different diseases. Three awards for a total of 92 drug analyses published in 2017 is a very low success rate. And for the third year running, none of the drugs examined offered a therapeutic advance worthy of a Golden Pill/Pilule d'Or.

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