

Issue 17: September 2024

4 new drugs approved this month for paroxysmal nocturnal haemoglobinuria!

This newsletter summarises final decisions from HTA bodies around the world from published reports in September 2024.

Good news for:

Danicopan, approved with orphan drug status for paroxysmal nocturnal haemoglobinuria in Germany.

Exagamglogene autotemcel, approved for transfusion-dependent beta-thalassaemia in England.

Toripalimab, approved for nasopharyngeal and oral squamous cell carcinoma in Austria.

Mixed news for:

Pegcetacoplan, approved for paroxysmal nocturnal haemoglobinuria in Germany but not in Scotland and Brazil.

Futibatinib, approved with special conditions for advanced cholangiocarcinoma in England but not in Germany.

Zilucoplan, approved for generalised myasthenia gravis in Australia but not in Scotland.

Bad news for:

Fezolinetant, not approved for vasomotor symptoms in menopausal women in Germany.

Ponatinib, not approved for chronic myeloid leukaemia in Brazil.

Tislelizumab, not approved for Squamous cell carcinoma of the oesophagus in France.

“

39 Approvals

10 with special arrangements
3 with orphan drug status

“

17 Not approved

5 Excluded

4 Excluded

“

12 Evidence reviews

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Pharmacological Technologies



Wales
AWTTC 5180

Blood

Paroxysmal nocturnal
hemoglobinuria
Crovalimab



England
NICE TA1000

Blood

Paroxysmal nocturnal
haemoglobinuria
Iptacopan



Germany
IQWiG G24-12

Blood

Paroxysmal nocturnal
hemoglobinuria
Danicopan



Germany
IQWiG G24-13

Blood

Paroxysmal nocturnal
hemoglobinuria
Pegcetacoplan



Wales
AWTTC 4537

Blood

Paroxysmal nocturnal
hemoglobinuria
Danicopan



Scotland
SMC 2715

Blood

Paroxysmal nocturnal
hemoglobinuria
Pegcetacoplan



England
NICE TA1003

Blood

Transfusion-dependent
beta-thalassaemia
Exagamglogene
autotemcel



Brazil
CONITEC

Blood

Paroxysmal nocturnal
hemoglobinuria
Pegcetacoplan



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Pharmacological Technologies



Brazil
CONITEC

Blood
Paroxysmal nocturnal
hemoglobinuria
Pegcetacoplan



Brazil
CONITEC

Cancer
Neuroblastoma
Betadinutuximab



Austria
AIHTA 189

Cancer
Advanced non-small cell
lung cancer
Amivantamab,
carboplatin, pemetrexed



France
HAS AP368

Cancer
Acute lymphoblastic
leukemia
Blinatumomab



Austria
AIHTA 190

Cancer
Advanced non-small
cell lung cancer
Atezolizumab



Scotland
SMC 2667

Cancer
Low-grade and high-
grade glioma
Dabrafenib



Wales
AWTTC 4485

Cancer
Advanced systemic
mastocytosis
Avapritinib



Scotland
SMC 2669

Cancer
Relapsed/refractory
multiple myeloma
Elranatamab



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Austria
AIHTA 187

Cancer

Advanced non-small cell
lung cancer
Encorafenib, binimetinib



Scotland
SMC 2664

Cancer

IDH1-mutated
cholangiocarcinoma
Ivosidenib



England
NICE TA1005

Cancer

Advanced
cholangiocarcinoma
Futibatinib



Brazil
CONITEC

Cancer

Ovarian cancer
Niraparib



Germany
IQWiG A24-66

Cancer

Cholangiocarcinoma
Futibatinib



Austria
AIHTA 188

Cancer

Urothelial carcinoma
Pembrolizumab,
enfortumab vedotin



Germany
IQWiG A24-81

Cancer

Relapsed and refractory
multiple myeloma
Idecabtagen vicleucel



Brazil
CONITEC

Cancer

Chronic myeloid
leukemia
Ponatinib



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Wales
AWTTC 3686

Cancer
Acute myeloid
leukemia
Quizartinib



France
HAS AP374

Cancer
Squamous cell
carcinoma of the
oesophagus
Tislelizumab



Brazil
CONITEC

Cancer
Thyroid carcinoma
Recombinant TSH



Austria
AIHTA 184

Cancer
Nasopharyngeal
carcinoma
Toripalimab, cisplatin,
gemcitabine



England
NICE TA1007

Cancer
Ovarian, fallopian tube,
or peritoneal cancer
Rucaparib



Austria
AIHTA 185

Cancer
Oesophageal squamous
cell carcinoma
Toripalimab, cisplatin,
paclitaxel



Scotland
SMC 2668

Cancer
Relapsed/refractory
multiple myeloma
Teclistamab



England
NICE TA1008

Cancer
Metastatic colorectal
cancer
Trifluridine-tipiracil,
bevacizumab



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Australia
TGA

Cancer
Grade 2 astrocytoma
or oligodendroglioma
Vorasicenib



England
NICE TA1002

Cardiovascular
Homozygous familial
hypercholesterolaemia
Evinacumab



England
NICE TA1001

Cancer
Marginal zone
lymphoma
Zanubrutinib



England
NICE TA1006

Endocrine
Type 2 Diabetes
Mellitus
Empagliflozin



Wales
AWTTC 5205

Cancer
Relapsed or refractory
marginal zone B-cell
lymphoma
Zanubrutinib



Germany
IQWiG A24-15

Endocrine
Vasomotor
symptoms
Fezolinetant



Austria
AIHTA 186

Cancer
Gastric or
gastroesophageal
junction adenocarcinoma
Zolbetuximab



France
HAS AP376

Endocrine
Chronic
hypoparathyroidism
Palopegteriparatide



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Brazil
CONITEC

Endocrine
Exocrine pancreatic
insufficiency
Pancreatin



England
NICE HST8

Genetic & Metabolic
X-linked
hypophosphataemia
Burosumab



England
NICE TA1004

Eye
Macular oedema
Faricimab



Scotland
SMC 2716

Genetic & Metabolic
Familial
chylomicronaemia
syndrome
Volanesorsen



Wales
AWTTC OW21

Gastrointestinal
Immune checkpoint
inhibitor (ICI)-induced
enterocolitis
Infliximab



England
NICE TA999

Genitourinary
Overactive bladder
syndrome
Vibegron



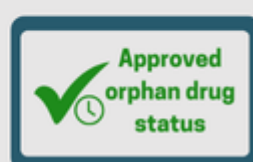
Wales
AWTTC OW22

Gastrointestinal
Immune checkpoint
inhibitor (ICI)-induced
enterocolitis
Vedolizumab



Germany
IQWiG G24-15

Infection
Bacterial infections
(multiple indications)
Cefepim/
Enmetazobactam



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Pharmacological Technologies



France
HAS AP395

Neurology
Metachromatic
leukodystrophy
Atidarsagene
autotemcel



Germany
IQWiG A24-67

Renal
Anemia due to chronic
kidney disease
Vadadustat



Australia
TGA

Neurology
Generalised
Myasthenia Gravis
Zilucoplan



Brazil
CONITEC

Respiratory
Severe COPD
Beclomethasone
dipropionate, formoterol
fumarate dihydrate,
glycopyrronium bromide



Scotland
SMC 2717

Neurology
Generalized
myasthenia gravis
Zilucoplan



Brazil
CONITEC

Respiratory
Severe COPD
Fluticasone furoate,
umeclidinium bromide,
vilanterol trifenate



France
HAS AP390

Renal
Primary IgA
nephropathy
Sparsentan



France
HAS AP378

Skin
Dystrophic
epidermolysis bullosa
Beremagene geperpavec



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Pharmacological Technologies



Germany
IQWiG A24-64

Skin
Hidradenitis
suppurativa
Bimekizumab



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Non-pharmacological Technologies



Brazil
CONITEC

Cardiovascular
Advanced left
ventricular heart failure
Left ventricular assist
device



England
NICE IPG793

Musculoskeletal
Chondral knee defects
Single-step scaffold
insertion



Argentina
IECS

Surgery
Benign tumours
Stereotactic
radiosurgery



Argentina
IECS

Surgery
Benign tumours
Stereotactic
radiosurgery



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Diagnostic Testing



South Korea
NECA PV-2024-11

Cardiovascular
Pulmonary embolism
Artificial Intelligence



Argentina
IECS

Cardiovascular
Cardiovascular risk
assessment
Coronary calcuim score



Argentina
IECS

Cardiovascular
Cardiovascular risk
assessment
Coronary calcuim score



South Korea
NECA HTA-2024-35

Mental Health
Schizophrenia spectrum
disorder
Korea-Polyenvironmental
Risk Score



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Other Guidance



Canada
CDA HC0033

Blood
Immune
Thrombocytopenia
Eltrombopag,
Romiplostim



Spain
AETSA

Cardiovascular
Ventricular arrhythmias
Implantable
extravascular
defibrillator



Spain
AQuAS

Cancer
Breast, lung and
colorectal cancer
Liquid biopsy, Next-
generation sequencing



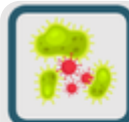
Canada
CDA OS0005

Endocrine
Type 2 Diabetes
Mellitus
Semaglutide



Spain
AQuAS

Cancer
Cancers
Liquid biopsy, Next-
generation sequencing



Canada
CDA HE0054

Infection
Pneumococcal infections
Pneumococcal conjugate
vaccines



Spain
AQuAS

Cancer
CNS Tumours
Next-generation
sequencing



Spain
AQuAS

Neurology
Frontotemporal dementia
Neuropsychological
tests



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Other Guidance



USA
HIQA

**Other
Migraine**

Cognitive behavioral therapy,
relaxation training,
mindfulness-based treatment



Evidence
review



USA
HIQA

**Other
Chronic pain
Cannabis**



Evidence
review



Canada
CDA RC1548

**Other
Chronic wound healing
Continuous Diffused
Oxygen**



Evidence
review



Canada
CDA RC1545

**Other
Pain management
Methoxyflurane**



Evidence
review

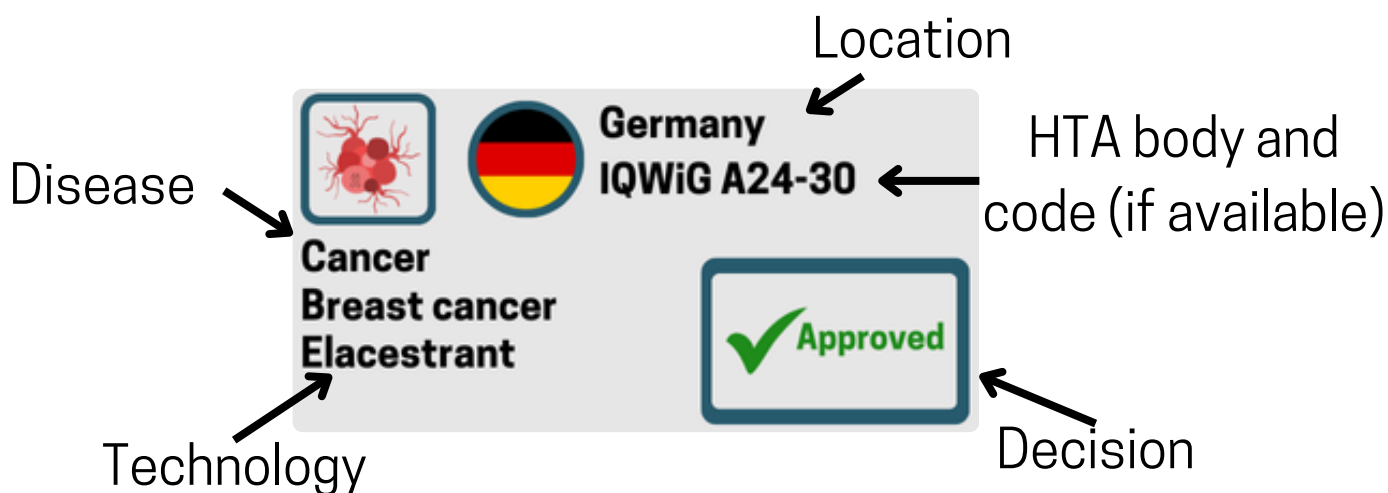


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Further Information

HTAngel is designed to help keep you up to date with the latest HTA decisions around the world. Below is a breakdown of the tiles in the newsletter.



At Crystallise we aim to provide an innovative approach to processing information, using software tools to automate processes that are slower and less accurate when done by humans. Our approach provides clients with greater transparency and scope than they could find for the same budget elsewhere. We work with a network of experts and organisations that help us support clients efficiently and effectively, and are proud of our flexible and ingenious way of solving their problems.

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To speak to us about our services contact contact@crystallise.com

