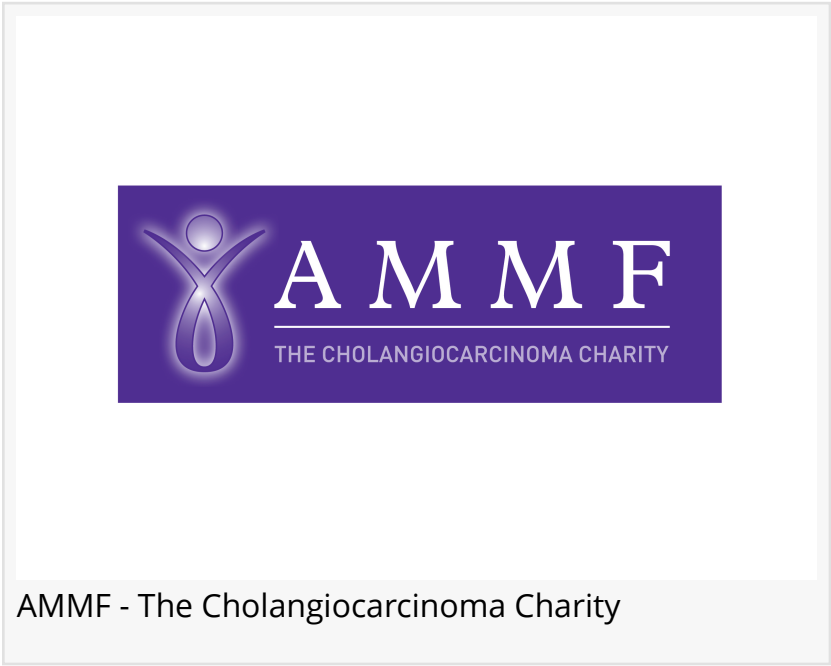
UK's NICE gives green light for pemigatinib (Pemazyre®) the first targeted therapy for those with cholangiocarcinoma

A potentially life-extending oral treatment for some people with cholangiocarcinoma, will now be available following recommendation by the UK's NICE today.



AMMF - The Cholangiocarcinoma Charity

AMMF, ENTERPRISE HOUSE, BASSINBOURN ROAD, STANSTED, ESSEX, UNITED KINGDOM, July 22, 2021 /EINPresswire.com/ -- AMMF, the UK's first and only cholangiocarcinoma charity is excited by an announcement made by Incyte Biosciences UK that a potentially life-extending oral treatment for some people with the rare and devastating liver cancer, cholangiocarcinoma, will now be available for routine use following recommendation by the UK's National Institute for Clinical Excellence (NICE) today.

Pemigatinib (Pemazyre) is the first ever treatment indicated specifically to treat adults with cholangiocarcinoma (bile duct cancer) when the cancer cells have an abnormal form of a receptor (target) called FGFR2 on their surface. Pemigatinib is used where cholangiocarcinoma has progressed after treatment with at least one systemic therapy (chemotherapy).

The active substance in pemigatinib belongs to a group of medicines called protein kinase inhibitors and works by blocking the activity of receptors called fibroblast growth factor receptors (FGFRs). Abnormal FGFRs are found on the surface of cancer cells and are involved in the growth and spread of the cancer. By blocking their activity, pemigatinib reduces the growth and spread of the cancer.

New treatment marks a major milestone in the fight against cholangiocarcinoma
Patients with cholangiocarcinoma - often referred to as one of the 'Cinderella' cancers because of its neglected status in regard to awareness and available treatments – are frequently diagnosed

too late to benefit from surgery, currently the only potentially curative treatment. In the UK around 2,430 people are diagnosed with cholangiocarcinoma every year1. Around 70% of these are diagnosed with unresectable, locally advanced, or metastatic disease, and these patients have an estimated 5-year survival rate of less than 10%2. Not only is pemigatinib the first ever treatment approved specifically for this devastating cancer in the UK and Europe, it is also the first approved targeted therapy for those with locally advanced or metastatic cholangiocarcinoma who have the FGFR2 fusion.

Helen Morement, CEO, AMMF

More research is desperately needed for this neglected and devastating cancer

As Helen Morement, CEO of AMMF, the UK's first and only cholangiocarcinoma charity explains, "This historic decision by NICE is warmly welcomed by AMMF and the patients and clinical communities we support. It gives hope to those eligible cholangiocarcinoma patients who will be able to access the first ever targeted treatment for this most challenging of cancers."

She continues, "Over the past 20 years we have tirelessly campaigned for more research and awareness of this neglected cancer on behalf of patients and their loved ones. This NICE decision will not only give eligible patients in England and Wales access to an alternative to chemotherapy, but it opens the door for cholangiocarcinoma patients to have molecular testing. Molecular testing signals a new dawn in our understanding of cholangiocarcinoma, bringing us one important step closer to finding further new treatment options for this devastating cancer."

"Cholangiocarcinoma is an aggressive cancer with a poor survival outcome so the availability of pemigatinib, the first targeted treatment option for those with this cancer who have an FGFR2 gene fusion, marks this as an important day for all diagnosed with cholangiocarcinoma," said

Professor John Bridgewater, Professor of Medical Oncology, UCL Cancer Institute, University College London Hospitals NHS Foundation Trust.

Professor Bridgewater adds, “In the clinical study which sought to evaluate the efficacy of this treatment for cholangiocarcinoma patients with an FGFR2 gene fusion, 82% gained a clinical benefit from pemigatinib3. I believe it is vitally important that all newly diagnosed patients receive timely and appropriate molecular testing to determine if they might be eligible for this treatment should they fail at least one line of systemic treatment.”

Patients’ reaction to latest news
English fashion and textile designer Dame Zandra Rhodes DBE, RDI, who was diagnosed with cholangiocarcinoma during lockdown and is campaigning to raise awareness of this disease, says, “It is important that people are aware of cholangiocarcinoma and its symptoms. I had never heard of this type of cancer before I was diagnosed and did not know what to expect. I am delighted to hear the news about the availability of a new treatment that may extend the lives of some people with this devastating disease. It gives me enormous comfort and will bring hope to people living with cholangiocarcinoma and their families.”

Married mother of three, Andrea Sheardown from Sandbach in Cheshire, believes the new targeted therapy raises hope. Andrea was given just six weeks to live by doctors when she was diagnosed with cholangiocarcinoma in 2015. Having had surgery that same year, followed by a gruelling six months of chemotherapy, Andrea is currently cancer free but is well aware that cholangiocarcinoma has a high incidence of recurrence. Andrea therefore welcomes pemigatinib as an important addition to the treatment armoury for herself and for others in a similar situation. As Andrea explains, “Thanks to this amazing news, now patients with cholangiocarcinoma who are tested and confirmed as carrying the FGFR2 gene fusion might derive considerable benefit from pemigatinib. It is game-changing - and potentially life-changing.”

The medication is available as tablets to be taken by mouth in three-week cycles consisting of two weeks where pemigatinib is taken daily followed by a week without the medicine. Treatment can continue for as long as the patient benefits from it and side effects are manageable.

Eligible patients with the FGFR2 gene fusion will be identified through molecular testing by their healthcare team. NICE’s guidance will enable all eligible patients in England and Wales to have access to pemigatinib (Pemazyre) through the National Health Service (NHS).

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AMMF is the UK’s first and only cholangiocarcinoma charity. Please visit https://ammf.org.uk/ for more information.

For more information about Incyte Biosciences UK, please click here

For more information about NICE, please click here

Office for National Statistics and the regional cancer registries in Wales, Scotland and Northern Ireland, and using the latest data (annual average number of cases in the UK between 2013 – 2015) (https://www.cancerresearchuk.org/about-cancer/bile-duct-cancer/about)

2 Graham RP, et al. Hum Pathol. 2014;45:1630□1638

3 Abou-Alfa et al. Lancet Oncology 2020: 21(5)671-684
4 EASL 2020, Poster THU-508. https://easl.eu/wp-content/uploads/2020/07/Digital-ILC-Scientific-programme-2020-Post-COVID-19.pdf

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