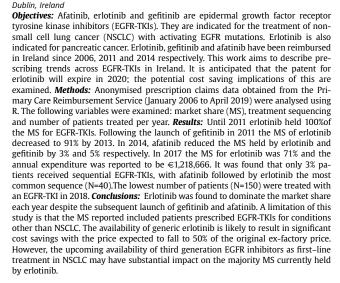
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implemented that will widen innovative contracting opportunities and thus facilitate access, but may also limit pricing. The ability to anticipate payer behaviour and navigate an increasingly complex access pathway will be critical for achieving optimal access for C> therapies.

PCN284

PRESCRIBING TRENDS ACROSS EPIDERMAL GROWTH FACTOR RECEPTOR TYROSINE KINASE INHIBITORS IN NON-SMALL CELL LUNG CANCER IN IRELAND





PCN285

THERAPEUTIC BENEFIT OF ORPHAN DRUGS IN ONCOLOGY: EVIDENCE AT THE POINT OF EUROPEAN MARKETING AUTHORISATION

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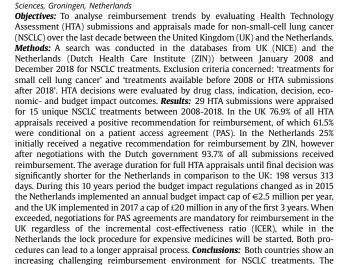
Objectives: We analysed evidence on therapeutic benefit of orphan oncology medicines approved in the past five years, at the point of marketing authorisation by the European Medicine Agency (EMA). **Methods:** Orphan oncology medicines authorised in 2014-2018 were identified from the EMA website. Data on trial design, overall survival (OS), progression free survival (PFS), adverse events (AEs) and health related quality of life (HRQoL) were extracted from the European Public Assessment Reports. Results: In the last five years, 23 products were approved in 38 rare oncology indications, eight of which were granted conditional marketing authorisation (CMA). Evidence was based on single-arm studies in 67% and 31% of CMA and full approvals respectively, with the remainder based on final or interim results of randomised controlled trials (RCTs). Included were three potentially curative advanced therapies for rare blood cancers which showed a significant increase in complete response rates compared to historical controls. Of those medicines evaluated through RCTs, prolonged PFS (1.2 to 19.9 months) was demonstrated in 64%, prolonged OS (1.0 to 49.2 months) in 36%, and increased number of serious AEs in 77%. Data on HRQoL was available in 64% of RCTs, of which 18% demonstrated improvement. All drugs were subject to post-approval evidence generation or surveillance. Conclusions: In rare oncology, particularly in potentially life-saving personalised treatments, demonstration of therapeutic benefit through RCTs is not always feasible, and nearly a half of approvals is based on single-arm studies. Most drugs evaluated in randomised settings demonstrated improvements in progression free survival, however there were frequent increases in serious adverse events. Evidence of overall survival gain was available in less than half of indications. Commitments to post-approval evidence generation were required for all medicines. Further efforts aimed at adaptive approaches to evidence generation are warranted, to ensure timely patient access and to stimulate innovation in medical practice.

PCN286

HEALTH TECHNOLOGY ASSESSMENT DECISIONS OVER THE LAST DECADE IN THE UNITED KINGDOM AND THE NETHERLANDS - A FOCUS ON NON-SMALL-CELL LUNG

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amount of positive recommendations for reimbursement were similar in both

countries, whereas the Netherlands showed a higher reimbursement rate after ne-

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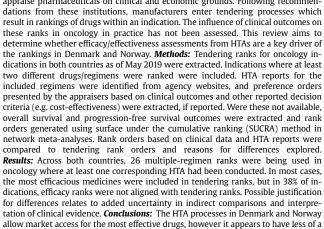
PCN287

gotiations.

THE ROLE OF EFFICACY AND EFFECTIVENESS ANALYSES IN HEALTH TECHNOLOGY ASSESSMENTS IN **DETERMINING TENDERING RANKS FOR ONCOLOGY DRUGS IN DENMARK & NORWAY**

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role in treatment prioritisation in oncology. Tendering presents one method for cost

containment for pharmaceuticals, but the impact this has on patient access to effi-

cacious treatments in oncology and other indications needs to be evaluated further.

PCN288

HOW IS METASTATIC MELANOMA TREATED IN THE PUBLIC HEALTH SYSTEM IN BRAZIL?: A CALL FOR CHANGE

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Objectives: - To undertake an exploratory analysis of the patients with metastatic melanoma and the treatment they received at SUS between the years 2015 and 2017. Methods: - We evaluated the data available at DATASUS, which were originated from the Ambulatory Chemotherapy Information Systems - SUS (SIASUS). In order to retrieve the data, we used the International Classification of Diseases C43 (the ICD for melanoma) and the period comprised between January 2015 and December 2017. These were then reviewed in order to be correctly assigned to treatment groups related to different levels of efficacy. We excluded those APACs that recorded ICD C43 but were related to supportive medications (such as bisphosphonates for bone







