

Early JCA Insights

Initial 4 Joint Clinical Assessments; all Orphan Drugs



Tovorafenib
(Omjeda®)

HTD: Ipsen

Indication: monotherapy in patients ≥ 6 months with paediatric low-grade glioma harbouring a BRAF fusion or rearrangement, or BRAF V600 mutation, after progression on ≥ 1 prior systemic therapies

No. of PICO: 8

Duration: 378 days

Assessors:
Ireland/ Germany



Lurbinectedin
(Zepzelca®)

HTD: Pharma Mar

Indication: maintenance treatment of adult patients with ES-SCLC whose disease has not progressed after first-line induction therapy with atezolizumab, carboplatin and etoposide

No. of PICO: 1

Duration: 384 days

Assessors:
Germany/ Portugal



Tarlatamab
(Imdylltra®)

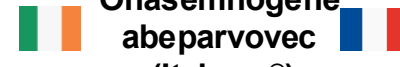
HTD: Amgen

Indication: treatment of adult patients with ES-SCLC, who require systemic therapy following disease progression on or after first-line treatment with platinum-based chemotherapy

No. of PICO: 7

Duration: 356 days

Assessors:
Germany/ Hungary



Onasemnogene
abeparvovec
(Iltvisma®)

HTD: Novartis

Indication: Treatment of 5q spinal muscular atrophy (SMA) with a bi-allelic mutation in the SMN1 gene in patients 2 years of age and older

No. of PICO: 12

Duration: 473 days

Assessor:
Ireland/ France

Some Areas for Discussion

Initial 4 Joint Clinical Assessments

 **Tovorafenib
(Omjeda®)** 

- High unmet need; Pediatric oncologic condition
- Limited data set with single arm trial only
- Inconclusive indirect comparisons



How to balance risk of Type 1 and Type 2 error in such condition?

 **Lurbinectedin
(Zepzelca®)** 

- Life threatening condition
- Small benefit in OS



How to handle different outcomes @ various data cuts?

 **Tarlatamab
(Imdylltra®)** 

- Life threatening condition
- Benefit across various outcome domains incl OS



How to consider subsequent therapies

Single out individual comparators in multi-comparator arms

 **Onasemnogene
abeparvovec
(Iltvisma®)**

- 3 licensed innovative medicines already available
- Lack of H2H comparison



Relevance / validity of indirect data

EMA and EU HTA have different perspective on extrapolation

And for all: how to manage Plethora of PICO schemes
 how to ensure meaningful clinical and patient input