Brukinsa® (zanubrutinib) – New orphan indication

- On September 1, 2021, BeiGene announced the FDA approval of Brukinsa (zanubrutinib), for the treatment of adult patients with Waldenström's macroglobulinemia (WM).

- Brukinsa is also approved for the treatment of adult patients with mantle cell lymphoma (MCL) who have received at least one prior therapy.

- The approval of Brukinsa for the new indication was based on ASPEN, a two-cohort, randomized, active control study in patients with MYD88 L265P mutation WM. Cohort 1 included 201 patients who were randomized to Brukinsa or Imbruvica® (ibrutinib) until disease progression or unacceptable toxicity. The major efficacy outcome was the response rate, defined as partial response (PR) or better based on standard consensus response criteria from the International Workshop on Waldenström's Macroglobulinemia (IWWM)-6 criteria. An additional outcome measure was duration of response (DOR).

    — Based on either IWWM-6 response criteria (standard or modified), the response rate was 78% with Brukinsa (95% CI: 68, 85) or Imbruvica (95% CI: 68, 86).
    — The event-free DOR at 12 months was 94% with Brukinsa (95% CI: 86, 98) vs. 88% with Imbruvica (95% CI: 77, 94).

- In cohort 2, patients with MYD88 wildtype (n = 26) or MYD88 mutation unknown (n = 2) WM received Brukinsa. Response using IWWM-6 or modified IWWM-6 was seen in 50% of patients (95% CI: 29.9, 70.1).

- The recommended dose of Brukinsa for both of its indications is 160 mg taken orally twice daily or 320 mg taken orally once daily until disease progression or unacceptable toxicity.