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Brincidofovir Development Update

- Brincidofovir (BCV) remains the only broad-spectrum antiviral in development, with antiviral activity against all five families of dsDNA viruses
  - Focus on patients and infections with highest unmet need: AdV, CMV, smallpox and BKV
- Oral BCV development continues for short course therapy
  - AdAPT: pre-emptive oral BCV for pediatric HCT recipients with adenovirus infection
  - Completing rabbit/mouse poxvirus models under Animal Rule
- IV BCV may avoid GI side effects, allow longer-term dosing
  - Multiple ascending dose study to complete end 2017
  - First studies in HCT patients with adenovirus viremia planned for Q1 2018
  - Proposed clinical plans include:
    - Dose-range finding study of IV BCV for treatment of CMV
    - Multi-viral prevention in high-risk adult and pediatric HCT recipients
    - Treatment of BK viremia in HCT or renal transplant recipients

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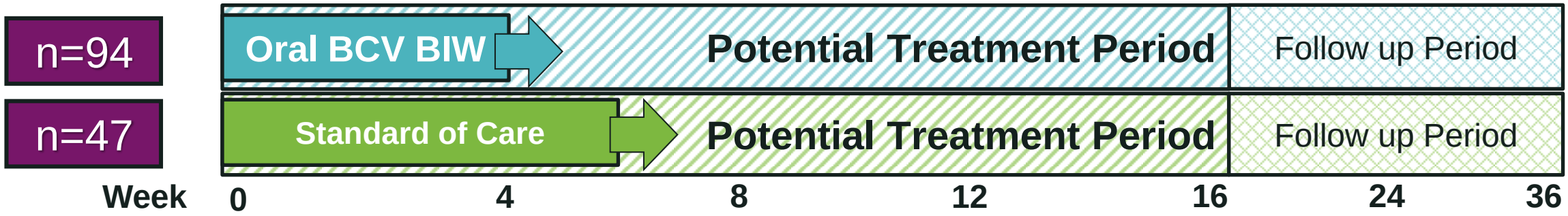
AdAPT: Adenovirus after Allogeneic Pediatric Transplantation

- **Open label, comparative study** of Oral BCV vs. standard of care (SoC)
  - Inclusion: pediatric T-cell depleted allo-HCT recipients in 1st 100d of HCT with AdV >1000 c/mL
- **Short course pre-emptive therapy**: BCV (or SOC) administered until AdV cleared from plasma and confirmed (an additional 6 days of therapy)
- **Sample size: N=141 (2:1 randomization)**



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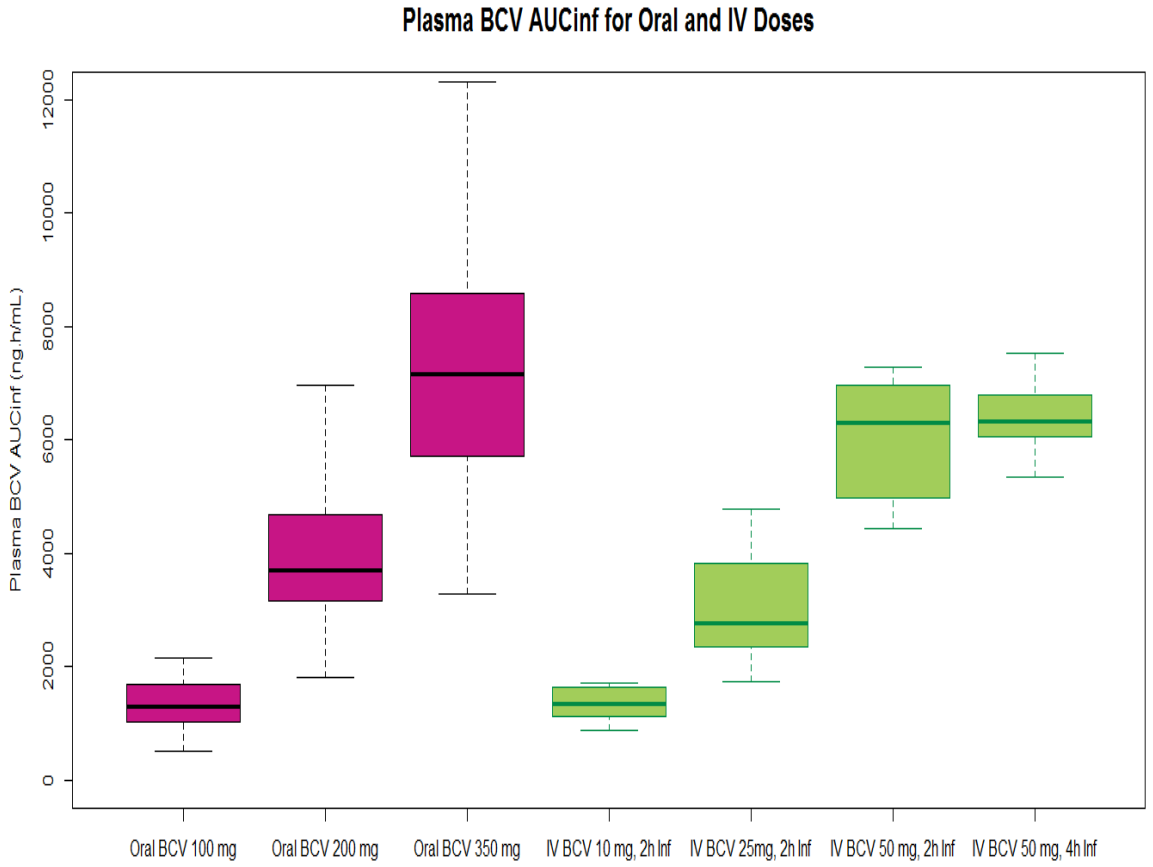
IV BCV Single Ascending Dose Study: Presented at ID Week 2017

- IV BCV 10 mg provided equivalent plasma drug levels to oral BCV 100 mg, which provided antiviral activity in prior studies
- IV BCV 10 mg and 25 mg were very well tolerated
  - No drug related AEs or significant lab changes
- IV BCV 50 mg infused over 2 hours was well tolerated
  - Only 1 subject reported loose stools
  - 50 mg over 4 hours resulted in higher rate of adverse events and ALT increases



Multiple doses and shorter infusion duration currently under study

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