"N-of-one Trials: Is This the Future of Oncology Drug Development?"

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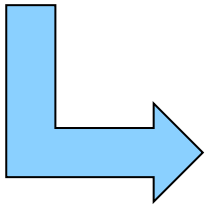
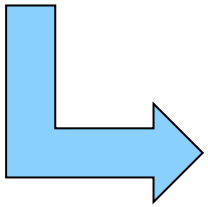
The evolution of drug development

- **Existing Paradigm**
  - OS: only endpoint for full approval
  - PFS with substantial clinical benefit, reproducible (x2)
  - Surrogates (used for accelerated approval)DFS, PFS, ORR, TTP



- **Challenges and transitions**
  - Single arm trials for approval
  - Small numbers/ rare tumors/rare genomic variants
  - Pan tumor approaches and Unknown primary



- **Future Paradigm: Precision Medicine**
  - Adaptive approach/adaptive design
  - Patient-centric approaches

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- N=1 can impact safety (e.g. SJS, TENS, PML etc)
- N=1 with longitudinal monitoring could be evidence of target activity
- N=1 could be informative for dose selection for combo trials

- Patient-centric trial where the molecular information determines the individualized treatment using pre specified algorithms
  - Gradual build up of cohorts of treatment groups.
  - Each patient considered individually

Doing now what patients need next