



Cracking the Code

Success stories in optimizing SAD/
MAD and NDA Enabling studies

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Decode the complexity of Phase 1 protocols

- **Subject burden**
 - Too many procedures, too many visits
 - Invasive procedures
- **Burden on the clinic staff**
 - More activities
- **Potentially compromise data integrity**
 - Collecting unnecessary data
- **Increased error rates/decreased quality**
- **Longer study durations**
 - Can delay submissions



Cryptogram

Consider adding in a FE portion to your SAD/MAD

- Will help alleviate any GI issues down the road caused by FE

Consider epTQT integration

- If there is not HERG signal in pre-clinical—highly recommend
- Can analyze immediately or collect and store
- Engage with renowned central cardiac lab during protocol development

Add patient cohort for early efficacy signals

- Only helpful if you have a specific indication in mind
- This data can be attractive to buyers
- Complicates overall execution of study
- Can greatly extend timelines for SAD/MAD trial



If timelines are critical



Consider overlapping MAD with SAD cohorts



If selling compound after SAD/MAD is priority

Are you Locking Yourself Out?



- Started SAD in half subject/half patient pop
- Moved into patients for MAD
- Scientifically clean
- SAD was hard to recruit—forced MAD to delay

- SAD and MAD in normal healthy population
- 2 cohorts of MAD in patients (rare disease)

- SAD and MAD in normal healthy population
- Last 2 cohorts of MAD in disease pop and then moved to OLE

- SAD and MAD all run in normal healthy population
- Had 6 primary objectives and 3 secondary objectives

Looking Ahead

Consider protocol timelines if you add patient/specialized pop to SAD/MAD

- Can DBL quicker if you carve out small patient cohorts into a separate protocol or only add 1-2 cohorts

Stick to clinical design basics

- 2 primary objectives
- No more than 2 secondary objectives
- Add in exploratory if needed

Things to consider in selecting your CRO of choice

- Keep SAD/MAD in one protocol or award each one to same CRO
 - Same core team for consistency
- CRO that can recruit the patient/specialized population
- CRO that has a manufacturing pharmacy on site
 - CMC is requesting 4-6 months lead time
 - Quick dosing decisions that can be completed on site



Worldwide
Clinical Trials

Thank you!

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