Advancing US Early Feasibility Studies for Innovative Medical Technology

University of Minnesota Design of Medical Device Conference
April 13, 2016
Vision

Clinical trial innovation has the potential to improve the safety and effectiveness of products being introduced into the market, reduce clinical trial cycle times and costs, and yield earlier access to beneficial innovative technologies for U.S. patients.

Priorities

• Address the infrastructure, data collection and pre- and post-market data requirements necessary to restore US leadership in clinical excellence
• Provide leadership in the use of innovative, efficient clinical trial designs
• Providing tools and methods to facilitate early feasibility studies in the U.S.
Our Speakers

• Carla Wiese, Policy Analyst for the Early Feasibility Program, U.S. Food and Drug Administration
  – CDRH Efforts to Enhance the Early Feasibility Study Program

• Angela Mallery, Regulatory Manager, North American Science Associates, Inc. (NAMSA)
  – EFS Strategy and Perspective

• Vicki Pearson, Vice President, Regulatory Affairs, Medtronic Aortic and Peripheral Vascular (APV)
  – MONA LSA Thoracic Branch Early Feasibility Pilot IDE

• Karim Benali, Vice President and Chief Medical Officer, Abiomed, MDIC Early Feasibility working group chair
  – MDIC Blueprint for Early Feasibility Study Success
CDRH Early Feasibility Study Program
Carla Wiese, FDA CDRH
Early Feasibility Study (EFS) IDEs

A Valuable Regulatory Tool for Medical Device Development

Carla M. Wiese
Policy Analyst for the Early Feasibility Program
Office of Device Evaluation
Center for Devices and Radiological Health

DMD Conference-Minneapolis
April 2016
Goal of the EFS Program

• Allow patients in the US the earliest access to potentially beneficial medical devices

• Encouraging innovation in the US by supporting the study of new technology
EFS Program Benefits

• Encourages development of high quality products
  ➢ Allows for device and procedure changes early in the product development process

• Results in high quality clinical data that can...
  ➢ demonstrate proof of concept which may be valuable to investors
  ➢ allow for faster US market approval by building on EFS knowledge
  ➢ be obtained for a device that has been used in compassionate use or emergency use cases and could support expanded device indications or a market application
  ➢ And more!!
Why the Focus?

- Clinical studies of novel technology are frequently conducted outside the US
- Devices may be approved outside the US only
- Device innovation may improve outside the US first

FDA is dedicated to supporting innovation in the US and enhancing patient access to beneficial technology
What is an EFS IDE?

IDE - Investigational Device Exemption

- Clinical study of an investigational device

EFS IDE - A standard IDE except...

- There are significant unknowns about how the device will perform
  - Device is generally early in development or
  - Device has a new intended use
- Small number of subjects in the clinical investigation
  - Initial indication of safety and/or effectiveness
  - Proof of concept

* EFS is an informal designation
## Types of IDEs

<table>
<thead>
<tr>
<th>EFS</th>
<th>Feasibility</th>
<th>Pivotal</th>
</tr>
</thead>
<tbody>
<tr>
<td>Small number of patients, &lt; 15 (approximate)</td>
<td>More patients than EFS</td>
<td>Number of patients determined by statistical needs</td>
</tr>
</tbody>
</table>

- **There are fundamental questions about device performance & safety**
- Device design may change.
- There may be limited nonclinical data available

Purpose of study can be...

- to demonstrate a proof of concept
- get a very early look at safety/efficacy
- examine human factors
- determine what design or procedure changes could optimize the therapy
- Determine patient characteristics that may impact device performance

Purpose of study can be...

- capture preliminary safety and effectiveness information and to adequately plan an appropriate pivotal study

Purpose of study can be...

- Demonstrate safety and effectiveness to support a marketing application

*note: not all of these are required for market approval*
What is CDRH doing to support EFS in US?

- Issued Guidance to outline FDA’s thinking on EFS and how FDA can be more flexible
- Established and trained EFS representatives in each division to assist sponsors and review teams
- Developed “CDRH-learn” modules focused on EFS
- Established FDA working groups to discuss alternative test strategies that are relevant
Key Elements of the EFS Guidance

• Doing the “Right Testing at the Right Time”
  ➢ Comprehensive testing during early phases of device development may add cost without significant return (some testing may be deferred)
  ➢ Informative nonclinical testing should be completed

• Unknowns and risk can be addressed by...
  ➢ Using clinical mitigations to provide patients with extra protection
  ➢ The use of more frequent/detailed reporting
  ➢ Informed consent recommendations
Key Elements of the EFS Guidance continued...

• Allows for timely device and clinical protocol changes
  - More changes can be made through 5-day notification rather than FDA approval
  - Contingent approval: approval of anticipate or proposed device changes can be obtained contingent on the completion of an agreed upon test plan and acceptance criteria

• Recommendations on pre-submission contents is provided
  - High quality submissions are important
Qualities of a Successful Submission
(for infrequent submitters in particular)

1. **Sponsor uses their resources:** Use FDA guidance documents & CDRH Learn Modules, communicates with FDA staff, seeks assistance with regulatory, nonclinical testing and clinical trial issues if needed

2. **Submissions are high quality**
   - Contents are well organized and navigable
   - High quality scientific discussion and evidence is provided
   - The sponsor is able to link together the information provided and tell the story of why an EFS is the right next step. (Why additional nonclinical testing will not be informative and a human clinical study is appropriate)
Qualities of a Successful Submission Continued...

3. Submissions are well planned

- Sponsor reaches out to EFS rep or FDA team to discuss plan
  - Informational meeting may be useful (for novel ideas in particular)
- Initial pre-sub includes...
  - Design concept, clinical context & rationale for early feasibility study
  - Description of the risks and how they will be addressed
  - Investigational plan information – high level look (who will be treated, what type of information you want to collect...)
- Additional pre-subs as needed (ex: if test requirements are uncertain/discuss clinical protocol)
- **IDE submission** contains all required information
Note:

- Use of pre-submissions to discuss the test plan and the clinical protocol...
  - Can be useful when the nonclinical testing needed is unclear, can agree upon the test plan that will support an IDE submission with FDA
  - May avoid the need to re-do expensive and time consuming testing
  - May help determine appropriate clinical mitigations, reporting requirements and the patient population for whom the benefit-risk profile supports inclusion into the EFS

Planning in Advance is Key
Qualities of a Successful Submission
Continued...

4. The decision to start human clinical work is well supported and explained

- There is a clear identification of potential risks & how they will be addressed
  - Nonclinical testing: Informative testing should be completed
  - Clinical mitigations strategies and appropriate reporting are proposed to protect patients - especially when nonclinical testing is uninformative

  ✓ Rationale is provided for why the plan is sufficient: Explain what can/can not be learned from bench tests/animal models & why any information to be leveraged is directly applicable to the study

  ➢ List which tests will be done to support the EFS versus which will be done to support a later study if applicable
Useful Documentation

- Devices approved under an investigational device exemption (IDE) are exempt from the Quality System (QS) regulation, except for the design control requirements under §820.30.

  - The design control requirements are basic controls needed to ensure that the device being designed will perform as intended when produced for commercial distribution.

  - Design control documents do not need to be submitted in the IDE but some of the information will support an EFS submission (risk analysis, design verification plan etc...)

  - Information about how the quality of the product and product performance is ensured will support the submission
FDA Review of Your File

1. The file is managed efficiently
   - EFS principles and guidance are followed.
   - Reviews are interactive and feedback is timely
     (Example: pre-sub feedback by day 45, meeting by day 60)
   - Review team and sponsor agree upon submission planning & best communication methods
   - Submission methods are appropriate (DES table useful or another tool?)
     - Improving EFS file tracking to better assist teams
     - On-going training

   = FDA efforts
2. The “right testing at the right time” principle is followed

- Agreed upon test strategy is appropriate and relevant leveraged information is considered

- Working groups have been established to discuss EFS specific strategies

  Working groups include biocompatibility, sterility, animal studies... Output: Document test considerations that are EFS specific

= FDA efforts
3. The decision to move to human use is appropriate and timely
   - Leveraged information that is valuable has been incorporated
   - The benefits and limitations of the nonclinical tests have been determined such that the decision point to move forward is clear
   - The team works with the sponsor to understand the link between potential risks, the sponsor’s test plan and the clinical mitigations

   ✤ Ongoing effort to find efficient ways to review test plans, define the unknowns and come to agreement on clinical mitigations strategies

   ✤ = FDA efforts
We Would Like to Hear from You
About your EFS Experience
(good or bad)

- Test requirements overly burdensome?
- Review team doing a great job?
- File progression is good/bad?

Contact me:

Carla Wiese
Policy Analyst for the Early Feasibility Program
301-796-0627
Carla.wiese@fda.hhs.gov
Other FDA Activities...

- **Working with CMS** to ensure coverage is appropriate by clarifying the process and criteria that CDRH and CBER use to assign CMS coverage categories in IDE approval letters.

- **Collaborating with Medical Device Innovation Consortium (MDIC)** on an EFS blueprint, which will be available for public comment on April 13: [mdic.org/EFSBlueprint](mdic.org/EFSBlueprint)

- **Working with academic centers** to provide tools to support physician investigators that want to develop and evaluate innovative devices.

- **Developing tools to support sponsors** that would like to submit high quality IDE submissions and achieve IDE approval.
Median Days to Full IDE Study Approval

- FY11: 442 days
- FY13: 215 days
- FY14: 101 days
- FY15: 30 days

- Use of pre-submissions
- Interactive communication
Are You Ready to Submit an EFS IDE?

- Planning ahead
- Read EFS guidance & Learn modules
- Engaged with an EFS rep or FDA team member to discuss a submission strategy
- Submitted pre-subs to establish a test plan & clinical protocol

We Look Forward to Supporting Your Effort!
Helpful Links

• Early Feasibility Study Guidance

• EFS CDRH Learn Modules
  http://www.accessdata.fda.gov/cdrh_docs/presentations/EFS/story.html

• Pre-Submission Guidance

• IDE Submission Suggestions
  http://www.fda.gov/MedicalDevices/DeviceRegulationandGuidance/HowtoMarketYourDevice/InvestigationalDeviceExemptionIDE/ucm046706.htm#reqele

• Design Controls Guidance
  http://www.fda.gov/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/ucm070627.htm

• Electronic Submissions Guidance
## EFS Representatives

<table>
<thead>
<tr>
<th>EFS PROGRAM LEADER</th>
<th>Names</th>
<th>Email</th>
</tr>
</thead>
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Sponsor Perspective: EFS Strategy and Perspective
Angela Mallery, NAMSA
EFS
Strategy and Perspective

Angela Mallery
NAMSA
Strategies and Practical Perspectives

- Learning the process
- EFS lens
- Realistic expectations
- My Advice
Learning the Process

- **EFS IDEs unique (not your traditional IDE)**
  - Does your device fit the definition/scope of the guidance
    - Have you exhausted bench/animal studies
    - Near final design
    - Why is this the best avenue to collect clinical and/or performance characteristics
  
  - **Structure of EFS IDE**
    - Background: design concept, clinical context, & plans for pivotal
    - Executive Summary: Summary tables and DES tables
    - Detailed Reports: data on testing, justification for leveraging from the DES tables

- The DES tables – example coming up
- Use the tools from the EFS guidance!
DES Tables

- Start at the beginning – list the positive attributes; and the possible failures/risks related to each attribute

- Mitigate – mitigate the risks; demonstrate the benefits outweigh the risks

- Leverage – How you mitigate risk = leverage previous versions and current versions of the device

- Make it easy to understand – color code it, stylize it, make it intuitive.
## DES Table – first half

<table>
<thead>
<tr>
<th>Column 1</th>
<th>Column 2</th>
<th>Column 3</th>
<th>Column 4</th>
<th>Column 5</th>
</tr>
</thead>
<tbody>
<tr>
<td>Device-Related Attribute</td>
<td>Potential Failure Modes</td>
<td>Potential Effects of Failure</td>
<td>Potential Clinical Effects of Failure</td>
<td>Device Design Information</td>
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<tr>
<td>Biocompatibility</td>
<td>Anaphylactic reaction</td>
<td>None</td>
<td>Anaphylaxis reaction</td>
<td>Selection of materials known to have minimal anaphylaxis response</td>
</tr>
<tr>
<td>Positive attribute</td>
<td>Failure</td>
<td>Effect of failure on the actual device</td>
<td>What harm comes to the patient</td>
<td>What did you design into the device?</td>
</tr>
<tr>
<td>Column 6</td>
<td>Column 7</td>
<td>Column 8</td>
<td>Column 9</td>
<td></td>
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<tr>
<td>----------------------------------------------</td>
<td>-----------------------------------------------</td>
<td>-----------------------------------------------</td>
<td>-----------------------------------------------</td>
<td></td>
</tr>
<tr>
<td>Leveraged Nonclinical Information (Previous Version and Justifications)</td>
<td>Supportive Clinical Information using Previous Systems</td>
<td>Completed Nonclinical Device Testing</td>
<td>Clinical Study Mitigation Strategies</td>
<td></td>
</tr>
<tr>
<td>Animal study (non-GLP) AS-101 conducted with version 1.0 of device show no AE</td>
<td>None</td>
<td>GLP animal study on v2.0</td>
<td>Procedure calls for continuous SpO2 monitoring for 24 hours.</td>
<td></td>
</tr>
<tr>
<td>Leveraging prototype or previous version of device ** not current version of device</td>
<td>Leverage any prior human use</td>
<td>Testing that will be conducted to support the EFS IDE with current version of device</td>
<td>How will you pay attention to the patients in your study</td>
<td></td>
</tr>
</tbody>
</table>
EFS Lens – how we view things differently

- Keep in mind the end goal is a **limited** human clinical trial

- Leveraging *previous version* of the device for *some* of pre-clinical data – what might you leverage?
  - Bench testing – limited samples sizes on final design with leveraged version of previous device
  - Biocompatibility testing – known materials?
  - Animal testing – represent safety?

- But…..Be prepared to conduct testing (leveraged data) will be necessary for subsequent Feasibility/Pivotal IDE
Realistic expectations

- Planning and organization are key – regulatory strategy, clinical strategy, what to leverage – what to test, and organizing the submission

- Not your traditional IDE – some different content/structure

- Pre-submission meetings – initial and follow ups; communicate with the FDA team to ensure the pre-subs do not extend timelines

- Help the review team at FDA
  - State often that conclusions based on EFS
  - State why mitigations are acceptable
  - State why certain items (such as SAP) are not included
  - Be organized
My advice

- Create a Road Map / Tables for consistency
  - Terms/ definitions/how data is discussed

- Previous versions of device
  - Compare versions & identify risks related to those differences

- Mitigation and current version of the device
  - Clearly identify when leveraging data & when testing has been conducted on final finished device
  - State why leveraging data is acceptable (each time)

- Pre-Submission meeting(s)
  - Engage in pro-active discussions
  - Work towards agreement
It is possible!

- **Plan**
  - 12 months of pre-submission meetings
  - And preparations

- **Manage expectations**
  - 6 months of IDE work
  - Interactive reviews with quick turn arounds
  - Goal of limited human trial

- **IDE approval!**
Sponsor Perspective: MONA LSA Thoracic Branch
Early Feasibility Pilot IDE
Victoria Pearson, Medtronic
MONA LSA THORACIC BRANCH
EARLY FEASIBILITY PILOT IDE
FIH IDE CLINICAL STUDY
Mona LSA:
- Modular stent graft
- Treat descending aorta aneurysms
- Preserve LSA patency
Left Subclavian LSA Artery (LSA)

40%
• Patients with limiting pathology
• Not good surgical candidates

Possible adverse effects
• Arm ischemia
• Paraplegia
• Stroke / Death

Possible Solution
• Single branch into LSA
• Preserve LSA patency
Novel Application to Address Unmet Need

Risk Mitigation:
- Known Materials
- In Vitro Bench Testing / Computational Modeling
- History LSA Coverage and secondary procedure

Procedure Verified:
- In Vivo Porcine Model
- Simulated Use Anatomical Models

Major differences b/w porcine and human models:
- Porcine heart movement more aggressive than human model
- No published data for human respiratory and cardiac motion at LSA

Need to Verify Initial Design and Procedure in Humans
- Patient characteristics
- Procedural steps / Human factors
APPROACH AND DEPLOYMENT

PILOT: Mona LSA stent graft accepted Jan 2012

Draft Guidance for Industry and Food and Drug Administration Staff

Investigational Device Exemptions (IDE) for Early Feasibility Medical Device Clinical Studies, Including Certain First in Human (FIH) Studies

DRAFT GUIDANCE
This guidance document is being distributed for comment purposes only.

Document issued on: November 10, 2011
APPROACH AND DEPLOYMENT

- MONA LSA Accepted in Pilot
- FDA Pilot Program Training
- First Implant
- FDA meeting to discuss Pre-Sub
- Comprehensive Pre-Sub
- IDE Submit
- IDE Approval

2011

- FDA Pilot Proposal Published
- FDA Face to Face meeting

2012

- Comprehensive Pre-Sub

2013

- IDE Submit
- First Implant
RESULTS

~ 2 year savings
- Just-in-time Testing
- Time to FIH implant shortened

PDP Process
- Utilized 5-Day Notices for minor protocol and design changes
- Re-evaluated and re-engineered internal processes

Impact
- ...potential pathway to total arch treatment solution
CURRENT STATUS

- Successfully completed EFS
- Have now moved from EFS to Feasibility study
- Developing strategy for initiating the pivotal
LESSONS LEARNED / EFS Process

- Communicate with FDA EARLY and OFTEN
- Use Pre-Submission Process
- Use Design Evaluation Strategy (DES) table
- Use 5-Day notices as appropriate
LESSONS LEARNED / Medtronic Project

- Physician Champions (n=2): Experienced Clinical Investigators Integrally involved in device design and implant procedure (n=2)
  - Important to address Physician training
  - Learnings important to design of pivotal clinical trial
  - Early FIH experience provided input to future patient selection criteria and implant procedure steps
  - Proactively work with FDA statisticians if you plan to leverage EFS or FS for your Premarket dataset
CONCLUSIONS

- Excellent opportunity for FIH in US
- Partnership with FDA and Clinicians to bring INNOVATION back to the US
MDIC Blueprint for Early Feasibility Study Success
Karim Benali, Abiomed
MDIC Early Feasibility Working Group Chair
Outline

• *Problem Statement*

• *Current Project Status*

• *Future Perspectives*
Necessary Ingredients to Medical Innovation

Capital

Knowledge

Medical Innovation

Ecosystem
Problem Statement

• There are over 6,500 medical device companies in the U.S.

• 80% of them have fewer than 50 employees.

• Companies need a favorable ecosystem to strive and grow

• Companies have moved clinical testing of novel devices overseas in response to an increasingly more uncertain ecosystem in the U.S.

• American patients and investigators are denied first access to novel technologies as a result of that.
Most of the Burden Occurs Early

It takes ~ 6.5 years and ~$36 Million in Investment Before the Start of the Pivotal Study

Perceived Challenges That Discourage Companies From Conducting Early Feasibility Studies in the US

- Regulatory process
- Ethics committee review cycle and processes
- Legal challenges (contracts, rights to patent)
- Cost (cost of insurance)
- Reimbursement
- Ethical / Safety evaluation: balance in risk/benefit determination
The Opportunity

- FDA CDRH has developed a new guidance and identified in their 2014-15 strategic priorities that they would like to see more early feasibility/first-in-human medical device studies in the US.

- Everyone (especially patients) would gain if industry leaders, FDA, payers, NIH and patient advocate groups developed a shared understanding of a balanced approach that will ensure the safety of the patient while promoting early access to innovation.

- In 2014, the MDIC CTIR Early Feasibility working group was tasked to identify the barriers to early feasibility studies “ecosystem” in the U.S. and based on the barriers identified propose possible solutions/tools to facilitate bringing these studies back to the U.S.
EFS Stakeholders

Patient

- FDA
- Payers
- Industry
- Patient Advocate Groups
- IRBs
- Scientific Societies
- Medical Community
- EFS Stakeholders
The EFS Working Group Roadmap outlines a timeline for initiatives across different stages of life:

**In-Utero (2014)**
- Build Team
- Op. mechanisms
- Define Roadmap

**Infancy (2015)**
- Interview stakeholders
- Survey
- Establish priorities

**Childhood (2016)**
- Process Improvement
  - Webinars
  - Workshop
  - EFS Blueprint book
  - Templates and Tools
  - Publicize

**Adolescence (2017)**
- Pilot program
  - 5-10 US EFS
  - Interactive feedback
  - More EFS in US

**Adulthood (2018+)**
- 80%+ of MDIC members do EFS in US

The roadmap includes a variety of activities and milestones aimed at advancing EFS initiatives across different stages of life, from conception through adulthood.
Blueprint for EFS Success in the U.S

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9. Insurance & Reimbursement
10. Patient Advocacy Groups
11. Conclusion
12. FAQs, Contacts & References

A Comprehensive Guide on How to Conduct Early Feasibility Studies in the U.S

Authors: MDIC EFS Working Group
(Draft released for public comment April 13.2016)

“The Guide I Wish I Had When I Got Started”

Draft available for public comment on MDIC Website starting 4/13/2016
http://mdic.org/EFSblueprint
What is it?
A guide that provides a bird’s-eye view and information on EFS with a practical approach, useful links and references to use.

For Whom?
Intended for sponsors, investigators and other audiences interested in EFS in the US.

What is not?
It’s not a substitution to any existing policy, practice, procedure, guidance or process of U.S. governing regulatory, legal, ethics, reimbursement agencies or institutions.

Milestones:
- April 13, 2016: Draft available for public comments on MDIC website
- End of public comments period: May 30, 2016
- End Q2, 2016: Final version released on MDIC website
Some Recent U.S Success Stories for Early Feasibility Studies

Early Feasibility Study IDEs *

- MITRALIGN Receives FDA Approval for Percutaneous Transapical Tricuspid Repair Early Feasibility Study
- August 11, 2015

"Through its application and close collaboration with the FDA, we are delighted to be able to bring our self-conforming, self-anchoring implant technology to US patients much earlier."
More Opportunities to Win for the Conduct of Early Feasibility Studies in the US*

70%+ of companies could “test” the new regulatory path for US Early Feasibility Studies… But some relative skepticism remains.

Would Your Company be Interested to Participate in US EFS program?

- Yes: 29.1%
- No: 20.3%
- Not sure. We will wait and see: 50.6%

* 2015 MDIC Survey (N=79)
Conclusion and Future Perspectives

• The conduct of Early Feasibility Studies in the US is feasible.

• Recent success stories exist and need to be publicized.

• MDIC will continue to work with the different stakeholders to identify tangibles actions that would help incentivize the conduct of EFS in the US to benefit the patients.
Acknowledgments

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Jessica Foley, Focused Ultrasound Foundation
Vicki Anastasi, ICON, plc
Terri Hinkley, Association of Clinical Research Professionals
Jonathan Batiller, Johnson & Johnson
Nancy Drake, NAMSA
Holli Hamilton, NIH
Petra Kaufmann, NIH
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Alison Alesi, Sterling IRB
Questions and Discussion
The MDIC draft Early Feasibility Blueprint is now available

http://mdic.org/EFSblueprint

We would appreciate your feedback!