An Introduction to the Medical Device User Fee Program: MDUFA V

Elias Mallis: Hello. Welcome to CDRH Learn, our program series for multi-media education for medical devices and radiological health products. I'm Elias Mallis, Director of the Division of Industry and Consumer Education. In this module, we'll provide you with an introduction to the Medical Device User Fee Program at CDRH, also known by its acronym, MDUFA V. The current user fee cycle runs from 2022 through 2027 and features several important initiatives that continue to build upon CDRH's implementation of user fees since they started over 20 years.

Your presenter for this module is none other than Dr. Jeff Shuren, Director of the Center for Devices and Radiological Health. Let's now get started with Jeff.

Jeff Shuren: Well thank you, Elias. One of the FDA's user fee programs is for medical devices, the Medical Device User Fee Amendments, or MDUFA Program. MDUFA just celebrated its 20th anniversary. Now when it started 20 years ago, the main focus was providing additional resources to the FDA so we could perform pre-market review in a more timely manner.

And over the next two iterations of MDUFA, we continue to refine the goals and the program. And then what MDUFA IV, we started to focus in on other areas important in pre-market review, and then MDUFA V truly takes it to the next level.

Under MDUFA V, we still have performance goals, and they're little tougher this go-round, and additional resources so we can conduct more Pre-Submission meetings. We know how important it is to developers to have the opportunity to talk with our reviewers and get their feedback.

And under the Pre-Submission Program, you can send in your questions and we'll provide a written response and the opportunity to meet within 70 days of receiving that request. And the MDUFA V Program covers so much more. So I'm going to take a few minutes and unpack it for you.

In MDUFA IV, we received funding to start a pilot that's called the Accreditation Scheme for Conformity Assessment, or ASCA. Now under that pilot, we would identify accreditation bodies who would accredit laboratories to conduct conformity assessment testing against international and national consensus-based standards that are recognized by the FDA for purposes of this pilot.

And the value of such an undertaking is that if we have high confidence in the laboratory who's doing the assessment, then we don't need to look at the raw data, we can just focus on a summary, which reduces a lot of time that we have to spend in our review. And for the company, they have higher confidence that the data they submit will be accepted by the FDA.

Well, it turns out the pilot was wildly successful, and as a result, MDUFA V now expands and it's going to turn it into a permanent program. So I would check it out, and if you have a device that's coming to us for review and you might be able to take advantage of one of those applicable standards, then I would certainly consider participating in the ASCA program.

MDUFA V also for the very first time provides funding support to expand our capabilities to advance international harmonization. We know that in the global marketplace, it becomes ever more important that we converge and optimally harmonize requirements across at least major countries so that developers have easier access into the marketplaces.

As part of our commitments, later this year, we're going to put out a draft strategic plan highlighting some of the important areas we're going to focus on over the next five years.

MDUFA V also provides funding and support to continue to expand our Patient Science and Engagement Program. We've been on an over a 10-year journey to better incorporate and understand the voice of patients in the work that we do.

Early on, we started to focus on methods to better understand and to quantify patient perspectives, such as the benefit-risk trade-offs that patients are willing to make around particular medical devices, what we call patient preference studies, as well as the development of patient-reported outcomes so that we're measuring things that are most important to patients. And with MDUFA IV, we were able to expand that program, and under MDUFA V, we're going to further invest in our capabilities.

Under MDUFA V, we're going to update some of our policies, like patient preference information guidance, and we're going to expand our activities around patient-generated health data to advance the use of remote clinical trials, and thereby also advance health equity by providing easier and greater opportunities for diverse populations to engage in evidence generation, particularly for clinical trials.

MDUFA V also continues to support our efforts on real-world evidence. Starting over a decade ago, we began to focus on creating more infrastructure and assuring that real-world data would be fit for purpose to be leveraged for regulatory purposes and includes issuing guidance that laid out the factors for consideration to determine whether or not real-world data and the evidence generated as a result could be used to inform FDA decision-making.

Part of those efforts was to establish what's called the National Evaluation System for Health Technology, or NEST. NEST is an attempt to build better infrastructure and greater efficiency so that real-world evidence can be generated and used more often within our ecosystem.

And NEST now is transitioning. It already has a series of data partners who then provide data consistent with a common data model, but now NEST is moving over to more of a general contractor approach where they are leveraging third parties as needed to provide additional capabilities such as around data extraction and data curation, platform for analysis, and then conducting those analyses to answer important questions for their customers, including those that may be important for the FDA, but also NEST is intended to answer questions that are important for providers and patients to assure safe use of devices, as well as to help inform payers around coverage and reimbursement decisions.

Under MDUFA V, we'll continue to support those activities, we'll update our real-world evidence guidance, and we'll expand and develop real-world evidence methods and policies for pre-market submissions, so we continue to increase the opportunities to leverage real-world evidence in our pre-market decisions as well as our post-market determinations.

MDUFA V also supports our digital health program. This, too, has been on a journey for over a decade. The current statutory framework for medical devices is 47 years old and it's not fit for purpose for many of today's modern technologies, particularly those that are software-based, like digital health technologies.

Recognizing this, over a decade ago, we began to revisit our policies and sort of right-size them under our current authorities to be better suited for digital health technologies. Under MDUFA IV, we received funding to help build a Digital Health Center of Excellence, and that Center has focused on looking at more innovative approaches to the oversight of digital health technologies.

For example, we ran a pilot on what's called pre-certification where we were looking at key capabilities of manufacturers and opportunities to leverage our understanding of those capabilities in lieu of performing the full deep dive into technologies on a technology-by-technology basis.

The Center has also put out several important policies-- more recently, final guidance on clinical decision support software, and also a growing focus on artificial intelligence and machine learning.

Under MDUFA V, we committed to issue a draft guidance for what's called pre-determined change control plans. In fact, we issued this draft guidance at the end of March. And here's the idea. That for certain kinds of modifications, a developer can identify those for the FDA and how they would go ahead and verify and validate those modifications. And if we agree that that's a good plan, then the developer can go ahead, make those modifications in the future as long as they follow the plan, and not have to come back to the FDA for another pre-market review.

We've also committed to issue final guidance on submissions for device software.

And finally, MDUFA supports a very exciting pilot for what's called the Total Product Lifecycle Advisory Program, or TAP. If you're a developer, trying to get to the market is really filled with pitfalls and traps. It's a game of chutes and ladders where you not only have to get through the FDA for marketing authorization, but coverage and reimbursement by payers, and ultimately adoption in the marketplace by providers and patients.

As I mentioned, MDUFA early on was very much focused on streamlining pre-market review, and yet we know that if you get everything right before you come in the door with your pre-market submission, the review of that submission should go very smoothly.

So what TAP is about is addressing those kinds of challenges-- essentially the valley of death going from concept to commercialization. TAP is intended to reduce the time and cost and increase the predictability of that valley of death to help spur more rapid development and more rapid and widespread patient access to safe, effective, high-quality medical devices of public health importance.

The way we go about it is three ways. First off, creating a new position, what we call the TAP Advisor. Now I mentioned that there's the opportunity today to seek advice from the FDA through Pre-Submission meetings, but those are kind of stage-gated. You send in your questions, and then within 70 days, we'll provide you with a response and we'll meet with you. If you have more questions, you throw them back over the transom and that process starts all over again.

The TAP Advisor, rather than just simply reacting to what companies are asking about, we'll engage proactively and strategically. We'll even independently reach out to the developer to start to identify what are going to be the key challenges and questions that need to be addressed. And then strategize on how best to do that to be successful.

Secondly, we're expanding our review capacity so that we have the opportunity to engage in much faster than that 70-day sort of stage-gate approach. Ultimately, we'd like to make this more real or near-real-time to be able to answer questions.

And then third, we know how important it is that developers understand the perspectives of providers and patients when they're designing their technology so that it best meets the value proposition for them, as well as thinking about the design on clinical studies so they're best suited for patient participation. Also, it's important to get the perspective of payers.

Therefore, under the TAP pilot, we essentially offer a menu of services, and that includes, if interested, the opportunity to be connected with the key patient groups, provider groups, and payers so that you can develop the best strategy for how to get from concept to commercialization and do that as efficiently as possible.

That TAP pilot was just launched in January, and as we committed to do, it was starting with just one office of health technology. So we're beginning with cardiovascular devices. And in the first year, we'll enroll up to 15. Already we've enrolled four companies and four technologies, so essentially, we're off to the races.

And you can see on the slide how this will progress over the course of MDUFA V as we expand to other technology types and have more participants in the program. To be eligible, you need to receive a designation as a breakthrough device and not have engaged with the program in a formal manner. So you've not had a Pre-Submission meeting.

And if you're eligible to be a part of TAP, I highly encourage you to do so because you'll have that opportunity for back-and-forth interactions to engage proactively and strategically, to be connected with other key groups if you're interested, and, in fact, to help mold that program, because we're innovative in our approach as well. We're going to keep iterating our prototype for TAP over the next few years. And then, of course, we'll assess its performance and that will help inform where we go in MDUFA VI.

I know I throw a lot of acronyms at you and you'll see those in the slides as well, so I gave you a handy dandy cheat sheet that you can use when you go back over them.

So finally, in summary, we have been incorporating the lessons learned from previous cycles into how the MDUFA program has evolved. And the user fees that we collect are going to allow us to meet the needs of our customers and drive innovation into the future.

Elias Mallis: Thank you for your attention to the CDRH Learn module on an “Introduction to the Medical Device User Fee Program: MDUFA V.” We encourage you to use other industry education resources we've developed especially for you. Of note, for comprehensive regulatory information, please contact CDRH's Division of Industry and Consumer Education. We look forward to helping you.

Thanks for watching this program and we'll see you next time.

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