NWX OD DIRS

Moderator: John Burklow
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Coordinator: At this time all participants are in a listen-only mode. After today's presentation we will conduct a question and answer session, and at that time you may press star then 1 on your phone keypad in order to ask a question.

Today's conference is being recorded. If you have any objections to this you may disconnect at any time. And now I would like to introduce your host for today's conference call, Mr. John Burklow. Sir, please go ahead.

John Burklow: Thanks very much (Brad). Hello everyone. I'm John Burklow, the Director for Communications at NIH. Welcome. I'll be the moderator for the call. Just to go through the format, Dr. Collins will make opening remarks and then we will open it up for questions and answers.

And I'm sure there'll be many people in the queue so I'll ask you to limit your questions to one per person. And so we can begin Dr. Collins.

Dr. Francis Collins: Thanks John. Good morning, good afternoon depending on where you are to all of you. I understand that more than 200 people on the phone, so clearly this is a topic of considerable interest.

And we're really pleased for the chance to spend some time going through the decisions that were put forward yesterday by the Scientific Management Review Board here at NIH that has significant consequences for the way in which we are planning to support therapeutics and translational medicine in the future, and also many other associated events that relate to that that I suspect you will want to ask about.

I do appreciate people taking time on very short notice to join the call. I apologize about the short notice but it's only been about six - well 28 hours since the decision was made by the SMRB to ask us to go forward, and we wanted to get as quickly as possible in a situation of being able to describe what the recommendations were and to listen to your comments and concerns.

I think it is fair to say that this is a remarkably exciting opportunity, but change is always something that can provide anxieties and breed rumors, and we feel the best antidote for those outcomes is information, and that's the goal here today is to try to provide as much of that as we can.

And I apologize because with so many people on the phone we probably won't be able in the time available to hear every question, but we'll do our darndest to try to be sure we address those that we can fit into the hour that we have together.

So I did want to though say a little bit at the beginning here about what is motivating this change and the opportunity it presents. It is the view of many of us that scientifically we are at a critical juncture.

Scientific advances, many supported by NIH, are providing new and very exciting insights into the molecular causes of disease at a dizzying rate,

whether you're talking about cancer or diabetes, whether you're talking about rare diseases or neglected diseases of the developing world, these insights are coming forward at an accelerated pace.

And many of these are potentially actionable, suggesting new approaches to prevention or to treatment. Yet I think we all would share the sense of frustration at how long it often takes from those original insights to be translated into a diagnostic or treatment that could be then applied clinically.

There are many reasons why this is a slow and inefficient process. For rare and neglected diseases the lack of economic incentives may mean that new therapeutic, potentially exciting ideas don't get attended to if we depend solely on the private sector and there's a need for academic investigators to play a larger role.

For common diseases many of the new targets that have been identified are of uncertain value, and so those also may lie dormant unless additional investments are made to explore them.

Even for projects that do enter the translational pipeline we all know high rates of failure are encountered at virtually every step, and there's a pressing need to really assess that pipeline as a scientific enterprise to try to see what could be done to improve efficiencies and to develop new technologies.

And this of course relates only to the so-called T1 phase of going from basic science to clinical trials. Obviously much effort also at NIH but effort that could perhaps be more thoroughly organized to look at what happens after approval of a compound in terms of post-marketing studies, comparative effectiveness research, pharmacogenomics, personalized medicine, behavioral research and many other related issues.

It is our view that we've arrived at a point where this circumstance requires an entirely new look at organization and not just an incremental tweak. And that after all was the motivation then to inspire the Scientific Management and Review Board, SMRB, to be asked to look at this.

If you're not familiar with the SMRB, it was put into place by the NIH Reauthorization Act in 2006 chaired by the distinguished Norm Augustine, populated by external experts and institute directors and charged with looking at NIH organizational structure to see whether we are in fact well positioned to take full advantage of new scientific opportunities.

But it seemed a very appropriate question to pose to them, are we in fact organized around translational medicine and therapeutics in the best way? A working group on translational medicine and therapeutics called TMAT was assembled last summer and asked to identify attributes and functional capabilities that would be needed for this kind of science to be maximally effective.

And that meant also to assess the NIH landscape for programs, for networks, for centers, components to include in this new structure and recommend their optimal organization.

SMRB charge was to try to complete this study by December, this month, so that if there was a recommendation about new organizational plans that could be folded into the FY12 budget request.

This is about the last moment to get any change of that sort into the administration's request in order to be into that cycle, in order to make it

possible to start something new at the beginning of FY12 which would be October 1 of 2011.

The team at working group took a very careful look at this, held a number of public meetings, sought input from many different perspectives and then yesterday made a formal recommendation to the Board, which the Board discussed and then voted upon.

And the Board specifically voted 12 to 1 for the following motion, that a new translational medicine and therapeutics center should be created as recommended by that group with components that I'll come to in a moment.

The Board also endorsed and supported a commitment by NIH to undertake a more extensive and detailed analysis to evaluate the impact of this new center on other relevant programs at NIH, especially those within the National Center for Research Resources, NCRR.

NIH is to report back their findings in that regard to the SMRB at its next meeting in February, but the SMRB was confident that the need for this new center, tentatively to be named the National Center for Advancing Translational Sciences, would be compelling and therefore they went ahead and endorsed that.

So NCATS as I guess its acronym would be pronounced if you were inclined to pronounce it has now been a formal recommendation to me as the Director.

Just a little bit of a technicality - before I can formally accept that recommendation, there's a requirement of consultation with the Congress and with the Secretary and those steps are rapidly getting underway.

But as you can tell from the fact we're having this call, I personally think the case is quite compelling for the need for this new center. And we felt it would be appropriate to begin right away to seek input from multiple stakeholders about the plan.

Now let me say a bit about what would go into this new center as imagined by the SMRB. The Molecular Libraries Program, which has been supported by NIH now for about five years and currently is located in the common fund and which offers investigators opportunities for assay development science and also for high throughput screening and medicinal chemistry of small molecule libraries, would move into this new center.

A program called TRND, T-R-N-D, which stands for Therapeutics for Rare and Neglected Diseases, which has been in place for about a year and a half and provides resources to move promising compounds through the pre-clinical phase, the so called valley of death, would also move into this new center.

A program called RAID, Rapid Access for Interventional Development, which provides such resources as GMP synthesis and animal toxicology, would also move into this center.

And a program called the Cures Acceleration Network which is authorized in the healthcare reform bill, and we expect will probably be appropriated for the first time in FY11, also focused very much on creative ways to move therapeutics and diagnostics through the pipeline, would also be a natural fit for the new center.

On top of that our relationship, which is increasingly strengthened with the FDA and includes a lot of shared intellectual discussions about creative

clinical trial designs, as well as now a regulatory science research agenda, would be supported to move into this particular new entity.

And perhaps most significantly in terms of the dollars involved, the network of 55 CTSAs currently located within NCRR recommended to move into the new Center for Advancing Translational Sciences with all of the capabilities they represent in a wide variety of clinical activities, as well as training and community outreach.

There was a significant discussion about whether the NIH Clinical Center should also be formally incorporated into this new entity, but after much discussion it was decided the complexities there were a bit too much to justify that change.

The Clinical Center will however begin to open its doors to extramural research projects, which is a good thing, and will have strong connections to this new NCATS entity but not in a way that the Clinical Center formally is moved within it.

I have talked primarily here about small molecules in terms of the therapeutic approaches, but the concept would also include biologics such as monocle antibodies diagnostics and perhaps even some focus on relevant biomarkers.

So that is what is definitely included as the proposal to go into this new center, but obviously that may not be the whole story. There are certainly other programs that could be a good fit with this emphasis on translation, many but perhaps not all of them located within the NCRR, for instance the comparative medicine program has been mentioned as one that might be appropriate to link up with this effort as have the primate centers.

And we need to take a thorough look at programs within NCRR to determine what would be the best fit here in terms of advancing the science. And in that regard I have asked Larry Tabak, who is the Principal Deputy Director at NIH and Alan Guttmacher, who is the Director of the NICHD to lead a task force effort to survey the portfolio of NCRR and make recommendations about what would be most appropriate in terms of where various programs should go.

I want to be clear that this is not an effort to dismantle programs - not at all. It's rather to identify new opportunities for scientific adjacencies that will make the whole even more than the sum of the parts.

All of these changes will be done in a way to protect the science that these programs represent. There may be of course some uncertainties about exactly what that means, but I did want to reassure those of you on the phone who are stakeholders of many of these programs, you know, that our goal here is to try to empower them and not to dismantle them.

And I hope that comes across loud and clear. At this point I'd like to ask Larry Tabak to say a word or two about the plans for this process of how this analysis of the NCRR portfolio will be undertaken. Larry?

Larry Tabak: Yes, thank you Dr. Collins. So as indicated a task force has been convened consisting of NIH Deputy Directors, Institute and Center Directors and Deputy Directors as well as Senior Program Officers from around the NIH.

And the idea will be to review all of the existing NCRR programs, as well as programs elsewhere within the NIH to get a sense for the NCRR programs of what makes the most sense to put into the new center, and what makes sense to move to other portions of the NIH.

These analyses will be heavily informed by content experts from the NCRR, and then once we have been able to draft a scientific framework we then will be reaching out to stakeholders such as yourselves to ensure that we get appropriate feedback and really get a full understanding of the specific programs that all of you are so heavily engaged in.

Ultimately informed by the NCRR, informed by the stakeholders a framework of science will be constructed and it will be that that will inform any administrative reorganizations that will be required. And so with that I'll turn it back to Dr. Collins.

Dr. Francis Collins: Great, and thank you Larry and thanks for your willingness to take on what is a really important part of this effort. I should say we are also reaching out to the PIs of the CTSA since obviously their programs are going to be affected in a significant way by this proposal of moving them into the new center.

And we had a phone call with them just about an hour ago and took a number of questions that they had proposed, which were all quite reasonable and helpful in the process of sorting this all out.

So if this all goes smoothly, although there are many steps obviously to be undertaken, the new center would be included in the FY12 budget and therefore be formally established on October 1, 2011.

We would undertake a national - international search for a director. That is not a hard-wired outcome at all. We would look for the very best person who has the kind of breadth and vision necessary for pushing forward this very challenging new set of scientific opportunities.

We want to be sure as this goes forward to try to address questions and respond as quickly as possible, as well as to try to squash any rumors that may fly around about what this is all about, because already we've seen that it's quite possible for those things to arise and quickly grab legs and walk away.

We are therefore putting up about - some time tomorrow a new Web site called NIH Feedback. You'll be able to get to it by going to the NIH home page where you'll see a link, or the direct URL is just feedback.nih.gov.

This site will allow you to make comments or pose questions, and we will use it also as a place to pose announcements about things that are happening for your information.

So it might be a good place to bookmark and see what's happening over the course of the next few weeks. So those were the main things that I wanted to put in front of you, and now we'd be glad to go to questions, so John.

John Burklow: Thank you very much Dr. Collins. (Brad), if you would queue up the first question that'd be great.

Coordinator: Absolutely. At this time if you would like to ask a question, please press star then 1 on your phone's keypad. If your question has been answered you may remove your request by pressing star then 2.

Once again please press the star 1 if you have a question at this time. Please stand by for our first question. Our first question will come from Mark Dresner. Your line is open.

Mark Dresner: Hi Dr. Collins. Mark Dresner with the University of Wisconsin.

Dr. Francis Collins: Hi.

Mark Dresner: I was curious about the meaning of the term that the NCATS would be established in October of ’11. Does that mean it will just exist as a ghost image until everything can be moved to it? Will the entire infrastructure be established in October of ’11?

Dr. Francis Collins: Well the goal will be to actually have it exist as more than a ghost. This is going to be a challenging undertaking administratively, and we have relatively little time to make this happen.

But the intention would be to have the entire administrative package in terms of the construction of the organizational chart, the decision about personnel who will be moving into this, the search for a new director and probably a deputy director and other critical efforts to have already been accomplished so that on that day there really is a center which has personnel, staff, capabilities of responding to questions, managing science and all that that entails.

As you can tell, that’s going to be a lot of work. We believe that starting now we can probably get that done.

John Burklow: Thank you very much. Next question please.

Coordinator: Our next question comes from Judy Glaven. Your line is open.

Judith Glaven: Hello Dr. Collins. This is Judy Glaven at Harvard Medical School. I’m actually here with a small group of faculty. We were wondering about how you’re thinking about incorporating and supporting basic science in this center as it relates to pharmacology, toxicology, technology development.

Dr. Francis Collins: Well great question and in fact I am one of those who feels that the future of translation will only be bright if it rests upon a very solid foundation of basic science, and that we cannot draw bright lines between those or we will regret it later.

So certainly much of the components of therapeutic development depend upon that, whether it's engineering producing some new technology platform for developing a diagnostic tool, whether it is new approaches to toxicology that do this more efficiently and less expensively than traditional methods, whether it's the medicinal chemistry which undergirds so much of what needs to happen in small molecules.

Now all of that effort has to be vigorously supported by NIH in order for this vision to come true, and I suspect significant components of that will need to be within this new center, especially the things I've just mentioned.

But of course we don't want this center to be isolated or a silo either, and again the potential at NIH of having connectivity between this and many other enterprises ought to make it even stronger.

I should have said all along of course there are translational efforts of major proportion in some of the institutes already, the Cancer Institute for instance, the Infectious Disease Institutes, the Neuroscience Institutes are already invested in many of these steps.

What we see though here is an opportunity to have a hub of activity to provide technology development and access to tools that maybe the smaller institutes don't easily have, and the larger institutes would be glad to have as a parallel activity that we can jointly learn from each other.

So I see this not as a, "Okay, we're emphasizing clinical and diminishing basic," but we're trying to pull the two together in a new and synergistic way.

John Burklow: Thank you very much. Next question please.

Coordinator: Our next question comes from Dallas Hyde. Your line is open.

Dallas Hyde: Hello Dr. Collins. This is Dallas Hyde, the Director of the California National Primate Research Center. What are your thoughts about the National Primate Research Center since many of our consortial activities with the Clinical Translational Science Centers and grants from other ICs involve translational research?

Dr. Francis Collins: So I'm going to ask Larry because this is one of the very important programs that he will be looking at as he reviews NCRR to comment on this.

Larry Tabak: Well, so we certainly acknowledge the importance of the program. The question that remains and has yet to be answered is what the ultimate fate will be, but we want to assure you and your colleagues that we are fully behind the program continuing.

It's just merely a question of administratively where would be the best fit, and in consideration of that we will of course take into account the scientific adjacencies that Dr. Collins has spoken to.

Dr. Francis Collins: And certainly we've already heard some strong arguments that primate research centers, because of their connection in many ways to translational efforts, ought to be looked at quite seriously as a possible additional component to move into this new center.

But that needs to be looked at with more thoroughness than we've had a chance to do so far.

John Burklow: Thank you very much. Next question please.

Coordinator: Our next question comes from Ken Kaushansky. Your line is open.

Kenneth Kaushansky: Hi Dr. Collins. This is Ken Kaushansky from Stony Brook. Is it envisioned that program project grants or multi-investigator grants that have an inherently translational nature to them be handled through this center, or would they still continue to go through the relevant institute?

Dr. Francis Collins: Are - you're asking about program projects that are already assigned to one of the other categorical institutes?

Kenneth Kaushansky: Either already assigned or newly envisioned grants.

Dr. Francis Collins: I think they will continue for the most part to go through the traditional pathways of the other institutes and centers as it relates to particular disease focus and orientation.

Again this center, although we're excited about it, is going to be even if fully funded up a very modest percentage of the overall NIH support, probably somewhere in the neighborhood of 1% or 2%.

And we expect that a great deal of clinical research, which is after all about 40% of the NIH budget, will therefore continue to go through the institutes who are very excited about continuing to do that kind of science.

Kenneth Kaushansky: Thank you.

John Burklow: Thank you. Next question please.

Coordinator: Our next question comes from Holly Falk-Krzesinski. Your line is open.

Holly Falk-Krzesinski: Hi Dr. Collins. This is Holly Falk-Krzesinski at Northwestern University CTSA. So far you have talked about programs that would be reassigned from other places within the NIH to become part of NCATS.

Is there an opportunity at this stage to propose important new activities or research areas that promote translational research, for example research on interdisciplinary research teams that are so essential to successful translational research?

Dr. Francis Collins: Well NIH will of course continue to explore all kinds of new opportunities to investigate areas of scientific importance. I would not say at this point there's any effort being made to identify what that sort of new list of possible RFAs might be.

Right now I think the goal is to try to figure out how to take the components that I've mentioned and put them together into a new entity. I might have also added, and your question sort of brings it up and I bet there are people on the phone wondering, NCRR of course has a number of programs that are in the process of soliciting new applications or maybe they've already been received or are about to have a deadline.

For the time being you should go ahead and respond to those just as you would have without this announcement. We have no intention here of slowing

down the progress that has already gotten underway with many of these programs.

Ultimately over the course of time they may move to other parts of NIH, but for the time being you should simply look at those and respond as you would have in any other normal circumstance.

Holly Falk-Krzesinski: Thank you.

John Burklow: Thank you very much. Next question please.

Coordinator: Our next question comes from Lauretta Gerrity. Your line is open.

Lauretta Gerrity: Yes, thank you. This is Lauretta Gerrity from the University of Alabama at Birmingham and also from the American College of Laboratory Animal Medicine.

I was interested in whether the PIs of the training programs and other NCRR funded projects would be included for input in this task force.

Larry Tabak: So yes - so this is Larry Tabak. Once we have a draft scientific framework we will reach out to all of the NCRR stakeholders, and certainly the training directors will be among that group. So we do look forward to discussing things with you in the future.

Lauretta Gerrity: Thank you.

John Burklow: Thank you very much. Next question please (Brad).

Coordinator: Our next question comes from (Denise Simmons). Your line is open.

(Denise Simmons): Yes, good afternoon and thank you for that very interesting talk on the new center. My question is one of inclusion as resources. Will there be a portal for commercialization and/or access to NIH researchers included in this?

Dr. Francis Collins: I'm not exactly sure of all the consequences or connotation of the portal. We certainly will see the relationship between the work going on in this new center and the private sector as critical.

What we don't want to do is to have NIH basically start competing with pharma or biotechs. That's not the goal. The goal here is to identify projects which might otherwise lie dormant for lack of economic incentive or scientific certainty.

And to de-risk them by the way in which NIH could then apply resources to move things along to the point where the project seems more attractive, and as soon as that attractiveness reaches the point where a company is interested in taking a project forward, we would strongly encourage then that that relationship be developed, and if it's a compound for instance it could be out licensed for the company to carry on to clinical trials.

There's some very interesting tech transfer issues here that relate to intellectual property and royalty arrangements, which we think will be fascinating to pursue and actually could be quite productive in terms of resulting in a win win for the NIH, for the company and another more important win for the public.

But much of that will need to be dealt with in a project-by-project way. I'm not absolutely sure that I got the full thrust of your question, but I hope that has some relationship to what you were trying to ask.

(Denise Simmons): Yes it does. Thank you.

John Burklow: Thank you very much. (Brad), next question please.

Coordinator: Our next question comes from Judith Van Houten. Your line is open.

Judith Van Houten: Thank you Dr. Collins for this opportunity and we applaud your vision and leadership in creating this new center. I'm the President of the IDeA PI Association and today I would just like to reiterate that the IDeA program probably does not fit well with this new center and would go to a new home, but that it's really critical that this program stay intact and not be divided up into multiple homes.

So my question is will we have some opportunity to make specific suggestions about alternative new homes for the IDeA program?

Larry Tabak: Yes, so again this is Larry Tabak. Absolutely. We certainly will reach out to you and your colleagues who run the IDeA programs. We recognize the importance of this program and we want to do nothing to harm it, so we will certainly be circling back with you.

Judith Van Houten: Thank you.

John Burklow: Thank you very much. Next question please (Brad).

Coordinator: Our next question is from Richard Larson. Your line is open.

Richard Larson: Hi Dr. Collins. This is Richard Larson. I'm from the University of New Mexico. I'm the PI on the CTSA and I'm also representing the COBRE and INBRE programs we have here.

My question was very similar to the one that was just asked. I was interested in both the future of the IDeA programs as well as the process going forward to determine what institute they would be housed in.

Dr. Francis Collins: So as Larry has mentioned that's a very important part of this close look at the NCRR program portfolio, and we all recognize how important the IDeA program has been to those states that have relatively modest amounts of NIH funding.

And certainly we'll take every bit of care here to try to assess what would be the best outcome, and I appreciate everyone's interest and I know Larry will take this with great seriousness along with his team.

John Burklow: Thank you very much. Next question please (Brad).

Coordinator: Our next question is from Gus Kousoulas. Your line is open.

Konstantin Kousoulas: Yes Dr. Collins, thank you very much for the opportunity to comment on the creation of the new institute. I'm a Professor here at Louisiana State University and also Vice President of the NAIPI representing the IDeA programs.

And perhaps I should emphasize the fact that this program has been essential to 23 states plus Puerto Rico in supporting science, specifically the COBRE

program is probably the most successful NIH program in turning young investigators with R1 competitive applications that have been very successful.

My question is relating specifically to the task force. Would the task force at NIH involve upper NIH officials representing the IDeA program from NCRR?

Larry Tabak: So again this is Larry Tabak. The intention is to work closely with our NCRR colleagues to help inform our deliberations as of course they are expert in each individual program that they are responsible for.

And then additionally of course as both Dr. Collins and I have mentioned, we will reach out to stakeholders extramurally with their input as well.

Konstantin Kousoulas: Thank you.

John Burklow: Thank you very much. Next question please.

Coordinator: Our next question is from (Camille Ungerbill). Your line is open.

Our next question is from Charles Mouton. Your line is open.

Charles Mouton: Yes, this is Charles Mouton calling from Meharry Medical College. I know NCRR has several programs that have filled the scientific data gap particularly for minority populations.

With the task force led by Dr. Tabak, will there be an opportunity to look for ways of sustaining and enhancing these efforts or programs like RCMI and SCOR?

Larry Tabak: So as part of the analysis a very major consideration will be by putting things in a new home potentially, will those scientific adjacencies lend themselves to increased efficiency and enhancement of the scientific opportunities?

So that will weigh very heavily in those deliberations and so again we will be reaching out to you and your colleagues in the RCMI programs for your best advice as well as we go forward.

Dr. Francis Collins: Dr. Tabak actually attended the RCMI meeting earlier this week and already had a chance to begin some of those conversations. This is a very important part of the deliberations.

John Burklow: Thank you very much. Next question please (Brad).

Coordinator: Our next question is from James Staros. Your line is open.

James Staros: Hi, Jim Staros at UMass Amherst, and I may just have missed something but the implication is that NCRR is going to distribute its programs elsewhere and it would dissolve.

That's part of the question and if that's so, what about the infrastructure grants that have been all clustered in NCRR? Maybe it's not so. Anyway that's the question.

Larry Tabak: Yes, so again this is Larry Tabak. As you know there are different flavors of infrastructure grants at the NCRR and we will decide, you know, what potential home would make the most sense for each of these programs.

We appreciate the importance of infrastructure on the former research teams so I truly appreciate it.

Dr. Francis Collins: Again let me just reiterate that the intention here is not to dismantle programs that currently reside within NCRR, but to figure out where is the best destination for each one of them that will strengthen their capabilities by the adjacencies that we can develop in that way.

John Burklow: Thank you very much. Next question please.

Coordinator: The next question is from (Maria New). Your line is open.

(Maria New): Hello Dr. Collins, this is (Maria New). I'm just calling to ask whether in this translational research there would be age groups separated, for instance fetuses, children, infants, or is this overall translational all age groups and all efforts would be included?

Dr. Francis Collins: So (Maria), I think the goal of this enterprise will need to involve a rigorous scientific peer review of what translational projects are likely to be successful, and of course that will factor into that scientific opportunity.

It will also factor into it other issues like is there a pressing need here, that is, we have a condition for which there is no current therapy. I can't tell you how that will shake out as far as age groups of the potential participants, although I think fetuses would probably not be high on the list considering all the complications there.

But certainly we do expect that this should provide an opportunity for pediatric research as well with all the appropriate constraints and concerns that relate to informed consent and ethical issues when we're talking about research on children.

(Maria New): I understand. May I ask one brief question? Will there be a way to direct applications to the translational center, or will they go through the regular study section route?

Larry Tabak: Yes, so this will - the expectation is, is that things will go through the typical study section route and through the typical referral guidelines from the Center for Scientific Review.

(Maria New): I understand. Thank you very much.

John Burklow: Okay, thank you very much. Next question please.

Coordinator: Our next question is from (Christopher Fox). Your line is open.

David Wong: Hello Dr. Collins, Dr. Tabak. This is David Wong here. I'm the President of the American Association of Dental Research. I'm here with my colleagues at the global headquarter in Virginia, Alexandria.

We have a question here we wanted to sort of - hope that you can share some insight for us is, is translating, you know, research portfolios, is in the future are there going to be opportunities where grantees could forward take their translational portfolio and interact with various constituents with the FDA for say pivotal clinical trials?

Are those consideration in the scope of this translational institutions that we're hearing today?

Dr. Francis Collins: Certainly part of the intention here is to provide that kind of capability because this is complicated and often difficult material for many investigators to try to get through, all of the regulatory hurdles that are necessary to get an

IND or to bring clinical trial data to the FDA in the format that they're used to seeing.

So yes, there would be an expectation here that this center would provide some of that support so that these kinds of translational projects would not get tied up in paperwork and bureaucracy if they had good science to push forward.

David Wong: Appreciate it. Thank you.

John Burklow: Thank you very much. Next question please (Brad).

Coordinator: Our next question is from Kent Lloyd. Your line is open.

Kent Lloyd: Hello, good afternoon Dr. Collins. Thanks very much for your comments. This is Kent Lloyd. I'm from the University of California at Davis. I'm the Director of Knockout Mouse Project Repository and one of the Directors of the Mutant Mouse Regional Resource Center, both funded by NCRR.

I was wondering if you could provide a little bit more in terms of details and specifics regarding your long-term vision in terms of metrics you'll use to measure and assess the success of the new center, such as in terms of funding, new directions, innovations, accomplishments either at the new center or at the institutes that may be impacted by this new initiative.

Dr. Francis Collins: So Kent, that's a great question and certainly one that we really have to look at very carefully in order not to over-promise what this center can accomplish.

All of you on the phone involved in biomedical research are aware of the long, complex and failure prone steps that are necessary to go from an idea to something that finds its way into clinical practice.

If this center is held up as being judged in its first two years by how many FDA approved drugs come out of this, it will most certainly fail because we all know that ain't going to happen on this timetable unless you have an exceptionally lucky circumstance of repurposing an already approved drug for a new application.

So we need to be managing those expectations and putting forward metrics that are much more realistic, but also measurable and hold ourselves in to ambitious standards.

Some of the kinds of metrics that have been talked about but nothing has really been settled as yet would include relationships that were made with the private sector in terms of in licensing or out licensing promising therapeutics, opportunities to hit certain milestones for particular projects.

Okay, you started with a compound that worked well in the laboratory. Have you now shown that it has adequate PK/PD? Perhaps along the lines of how many INDs can you imagine issuing or applying for in the space of the first five years let's say, and how many of those were approved?

What kinds of clinical trials were started that would not have been otherwise, especially Phase I and Phase II which could very well be conducted within the CTSAs or the NIH Clinical Center?

There are various other metrics we could talk about that are perhaps more traditional. Publications for instance ought to be a big part of this, but at the

moment this is very much a work in progress to try to decide what those should be.

We will need though to have those in advance of the center being set up so that as people are looking at it in its first few years, there will be a standard by which success can be measured.

John Burklow: Thank you very much. Next question please.

Coordinator: Our next question is from (Ang Sun). Your line is open.

(Jung Jiasan): Hi Dr. Collins, (Jung Jiasan) from the University of Oklahoma Health Science Center. I have a question related to the budget. Do you expect that Congress will approve additional budget for this new center?

If not, are you going to remove budget from the current institute center to this new centers? If so, how will it affect the current payouts?

Dr. Francis Collins: So I wish I could forecast that the Congress will decide to allocate new monies for this or for anything else at NIH. And the current budget climate though as you know, things are very severely constrained because of the economic situation in the country and the increasing concerns about the deficit.

So we are planning to stand this center up without the expectation of a big bunch of new money, because I think it would not be realistic to imagine that that is going to be coming forward.

I would say however that as we try to make the case for NIH's contributions to human health, that a new initiative of this sort resonates quite well with the administration and the Congress and with the American public.

And this is a good example of why I think even in difficult budget times we should continue to try to put forward new innovations. Going forward beyond the initial startup of this, just as with any other aspect of what NIH does we're going to have to make considered judgments about priorities.

I can't tell you how that will play out. I would say in the course of a five year plan it's very difficult to say which programs will be seen that need an additional boost and which will be seen as perhaps able to manage a little belt tightening.

That is a tough problem that we at NIH deal with all the time. It's obviously easier when you're in the middle of a doubling than when you're facing what could be at best flat purchasing power. But we will have to deal with that program by program on a scientific basis.

(Jung Jiasan): Thank you.

John Burklow: Thank you very much. Next question please.

Coordinator: Our next question is from Henry Lewis. Your line is open.

Henry Lewis: Good afternoon Dr. Collins. I want to first thank Dr. Tabak for coming on the floor in the university. I'm the RCMI program Principal Investigator there and we're currently attending the RCMI meeting here in Nashville, and for having a very open and candid discussion with us at our meeting here earlier this week.

My question is a follow up on the infrastructure, a question that was raised a little bit earlier. NCRR supports a wide range of underburdened biomedical research enterprise for the entire nation.

My question then is has there been talks given to how can you bring those other entities with - that are currently at NIH that are supportive of the infrastructure building of the biomedical research enterprise into what will be left of NCRR?

And will that be an opportunity for NCRR to continue the role that it is playing as a major supporter of biomedical research in the country?

Larry Tabak: Well, this is Larry Tabak and thank you for acknowledging my visit with you and your colleagues. It was enjoyable for me to meet with you all. So in terms of, you know, how this will play out the short answer is we don't know yet because we are just now in real-time beginning the analysis.

But in all candor it is more likely than not that the remaining programs at the NCRR not related to translational science will find new homes at other existing institutes and centers.

And we will do that in a way that will enhance and maximize, you know, the strength of the programs by taking into account what exists in their potential future home.

John Burklow: All right, thank you very much. We have time for a couple more questions.

Coordinator: Our next question is from Tryone Spady. Your line is open.

Tyrone Spady: Hi, this is Tyrone Spady from the Federation of American Societies for Experimental Biology. My question is in the context of the working group's discussions of the TMAT deliberations, several organizational options were mentioned, institute centers, programs, so, I mean, I guess based on Dr. Tabak's previous comment does that mean that there are no additional options for - no additional standalone options for the remaining components of the NCRR program?

Larry Tabak: So again this is Larry Tabak. The short answer is we don't know yet, but again the longer answer and trying to be as candid as possible, it is highly unlikely that you would keep the remaining programs together intact.

Frankly if you looked at what remains it really does represent a bit of a patchwork quilt, and for good reason different programs and needs emerged over time and they found themselves in the NCRR.

And we're very grateful for the stewardship of those programs over the intervening years, but given the opportunity to reassess I wonder if anybody would recreate what would be left and say, "This all makes perfect sense."

That's the type of discussion and analyses that our group will have to have over the next several weeks to come.

John Burklow: Thank you Larry. Next question please.

Coordinator: Our next question comes from Charles Wood. Your line is open.

Charles Wood: Yes, good afternoon Dr. Collins and I would appreciate a time and the opportunity for us to learn about the institute and also for us to have some questions.

I am from the University of Nebraska and I am the past President of the IDeA PI Association. It has been brought up earlier about the IDeA program and there are NCRR program that is remaining after CTSA is moved out.

My question is because it's a lot of interaction and actually enhancement of these programs, for example the IDeA, the RCMI and including SEPA, there's a lot of interactions between the program directors.

So if the - these programs are dismantled or dispersed in different institute, it might have some detrimental effect in the long run. So are there any considerations of moving them as a cluster so that it can maximize the benefit for each other? And what's the opportunity for them to grow as well? Thank you.

Dr. Francis Collins: So it's a great question, and again there is no intention to dismantle programs. What we are trying to do is to achieve effective adjacencies and not diminish ones that might actually already be quite productive.

And that's one of the things that Larry's group is going to need to look at. I've certainly heard similar concerns about the possible loss that would happen if for instance the IDeA program and the RCMI program were not next door to each other in terms of their staff support, and that will be an important consideration.

You mentioned the SEPA program, which also is one that we're quite interested in seeing have an effective and vigorous future. The SEPA program of course involving as it does educational efforts may need some other adjacencies also with the increased emphasis from the White House of the

importance of this, and certainly my personal interest as well in trying to see if NIH could do more in this regard.

So the trick will be to try to find the optimum mix there where you are increasing the muscularity of each of these efforts by the way in which you rearrange these various components.

I guess just because everything at some level is connected to everything else, whatever we come up with will probably not have the sort of perfect solution attribute to it, but we'll try to maximize the benefits of the overlaps as this careful analysis is done by Larry and Alan and their team. Okay (Brad), thank you.

John Burklow: Thank you. (Brad), we have time for one more question.

Coordinator: Our final question comes from (Valerie Montgomery Rice). Your line is open.

Keith Norris: This is Keith Norris from the RCMI Program Directors' Association, and first I want to again say thank you to Dr. Collins and Dr. Tabak, and again Dr. Tabak, thank you for visiting.

I don't want to be redundant with some of the questions regarding the future of the RCMI and IDeA programs, but given the fact that the minority rural and urban core of the major groups suffering from premature morbidity and mortality, and we're looking at how we can accelerate advances to this end.

I haven't heard much about translating therapeutic to clinical practice and how that will happen, and the potential role particularly of the programs such as RCMI and IDeA that could play a significant benefit in that aspect of translational science.

Dr. Francis Collins: So it's a good question. As I mentioned earlier the new center will end up being only a small proportion, a very small proportion of NIH and therefore a small proportion of what we invest in clinical research.

I certainly agree with the point you're raising about the importance of real world implementation research, especially in diverse settings for diverse populations.

I think NIH perhaps in the past has been accused of being a little bit less concerned about actually implementing the results of our research in the general public, as opposed to doing a very carefully controlled trial under a circumstance that might be hard to replicate in a real medical setting.

And I think we would like to very much resist that complaint about doing more of this sorts of research and that's something that's already I think getting some attention in the comparative effectiveness research arena.

So clearly that will be an important agenda and the RCMIs are in a very important position to play a role in that, as are the IDeA states because of access to populations in rural settings that are underserved.

Exactly how that plays out in terms of the formation of this new center is one of those things that will have to be considered amongst many other practical concerns about what makes the most sense to put together in one unit, and to what extent we have to also depend upon all of the components of NIH to work effectively together regardless of which organizational box they happen to be in.

I must say for me as the NIH Director this is - one of the highest priorities is to be sure that our whole is really much greater than the sum of our 27 parts, and that will be a big emphasis even as we seem to be creating a new part in this process at the center. So thank you for the question and John, shall I sum up here?

John Burklow: I think this is a good opportunity and if you have questions that you weren't able to ask today, we do have the Web site Dr. Collins mentioned that will be going up tomorrow and look for it on the NIH home page. So I'll turn it back over to Dr. Collins for closing comments.

Dr. Francis Collins: Well this has been a wonderful set of thoughtful questions from all of you, and yet I'm sure there were people still waiting in the queue who didn't get called upon.

And with more than 250 people on the phone I'm afraid there wasn't a whole lot of way we could handle every question, but I think many important issues were raised.

Again we want this to be the start of an ongoing conversation with important stakeholders, which are all of you. We will be with Larry and Alan's help trying to put together an initial view of what would make the most sense as far as these assignments of important programs.

And as that begins to take shape we will be sure to put it out there for comments and see with great seriousness what people think about the proposed plans.

Even though change can be unsettling and can certainly cause anxieties, I hope you all would share with me a sense of excitement about the scientific

opportunity that is driving all of this and the chance to do something that could be really quite beneficial for the American public, in fact the public of the whole world which is after all our mission and a mission that I know you share.

But thank you for your thoughtful questions and for your willingness to engage with us today and over the next few weeks as we together try to create a new framework for developing therapeutics that are going to make a big difference in our world.

John Burklow: Thank you very much everyone and I'll turn it back over to you (Brad).

Coordinator: Thank you for your participation on today's conference call. At this time all parties may disconnect.

John Burklow: Thank you.

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