

***** NOTICE OF GRANT AWARD *****

Issue Date:08/18/2004

Department of Health and Human Services
National Institutes of Health

NATIONAL CENTER FOR RESEARCH RESOURCES

Grant Number: 7 U54 RR019484-02 (Revised)
Principal Investigator: NEW, MARIA I. MD
Project Title: Natural History of Rare Genetic Steroid Disorders

MS JESSICA MOISE
DIRECTOR
GRANTS AND CONTRACTS OFFICE
MOUNT SINAI SCHOOL OF MEDICINE
NEW YORK, NY 100296574
NEW YORK, NY 100296574
UNITED STATES

Budget Period: 06/01/2004 - 07/31/2004
Project Period: 09/30/2003 - 07/31/2008

Dear Business Official:

The National Institutes of Health hereby revises this award (see "Award Calculation" in Section I and "Terms and Conditions" in Section III) to MOUNT SINAI SCHOOL OF MEDICINE OF NYU in support of the above referenced project. This award is pursuant to the authority of and is subject to terms and conditions referenced below.

Acceptance of this award including the Terms and Conditions is acknowledged by the grantee when funds are drawn down or otherwise obtained from the grant payment system.

Award recipients are responsible for reporting inventions derived or reduced to practice in the performance of work under this grant. Rights to inventions vest with the grantee organization provided certain requirements are met and there is acknowledgement of NIH support. In addition, recipients must ensure that patent and license activities are consistent with their responsibility to make unique research resources developed under this award available to the scientific community, in accordance with NIH policy. For additional information, please visit <http://www.iedison.gov>.

If you have any questions about this award, please contact the individual(s) referenced in the information below.

Sincerely yours,

Mary Niemiec
 Grants Management Officer
 NATIONAL CENTER FOR RESEARCH RESOURCES

See additional information below

SECTION I - AWARD DATA - 7 U54 RR019484-02 (Revised)

AWARD CALCULATION (U.S. Dollars):

Salaries and Wages	\$38,066
Fringe Benefits	\$9,706
Personnel Costs	\$47,772
Equipment	\$6,000
Supplies	\$52,500
Travel Costs	\$16,000
Patient Care (Outpatient)	\$40,000
Other Costs	\$74,431
Consortium/Contractual Cost	\$491,389
Federal Direct Costs	\$728,092
Federal F&A Costs	\$202,039
APPROVED BUDGET	\$930,131
TOTAL FEDERAL AWARD AMOUNT	\$930,131
AMOUNT OF THIS ACTION (FEDERAL SHARE)	+\$0

Recommended future year total cost support, subject to the availability of funds and satisfactory progress of the project, is as follows.

03	\$1,123,337
04	\$1,156,489
05	\$1,074,990
06	\$1,135,127

FISCAL INFORMATION:

CFDA 93.389
 Number:
 EIN: XXXXXXXXXX
 Document Number: URR019484B

IC/ CAN /	FY2003 /	FY2004 /	FY2005 /	FY2006 /	FY2007
OD/8421751/	930,130/	1,123,336/	1,156,488/	1,074,989/	1,135,126
RR/8422434/	1				
RR/8464629	/	1/	1/	1/	1

NIH ADMINISTRATIVE DATA:

PCC: CRT65 / OC: 41.4P /Processed: NIEMIECM 040815 0538

SECTION II - PAYMENT/HOTLINE INFORMATION - 7 U54 RR019484-02 (Revised)

For Payment and HHS Office of Inspector General Hotline Information, see the NIH Home Page at <http://grants.nih.gov/grants/policy/awardconditions.htm>

SECTION III - TERMS AND CONDITIONS - 7 U54 RR019484-02 (Revised)

This award is based on the application submitted to, and as approved by, the NIH on the above-titled project and is subject to the terms and conditions incorporated either directly or by reference in the following:

- a. The grant program legislation and program regulation cited in this Notice of Grant Award.
 - b. The restrictions on the expenditure of federal funds in appropriations acts, to the extent those restrictions are pertinent to the award.
 - c. 45 CFR Part 74 or 45 CFR Part 92 as applicable.
-

- d. The NIH Grants Policy Statement, including addenda in effect as of the beginning date of the budget period.
- e. This award notice, INCLUDING THE TERMS AND CONDITIONS CITED BELOW.

(see NIH Home Page at <http://grants.nih.gov/grants/policy/awardconditions.htm> for certain references cited above.)

Carry over of an unobligated balance into the next budget period requires Grants Management Officer prior approval.

Treatment of Program Income:
Other Research (Add/Deduct Option)

SECTION IV - NCRR ADDITIONAL TERMS AND CONDITIONS - 7 U54 RR019484-02 (REVISED)

This award is revised to adjust NIH accounting information only.

PREVIOUS SECTION IV - NCRR ADDITIONAL TERMS AND CONDITIONS - 7 U54 RR019484-02

This award may be revised downward if the actual expenditures reported by the former grantee, Weil Medical College of Cornell University, are greater than those estimated on the Relinquishing Statement.

This award is issued as a Cooperative Agreement and includes the Terms of Collaboration detailing the nature of the awardee's collaborative relationship with the Office of Rare Diseases Program Coordinator, NCRR Program Coordinator, the participating IC Program Officers, Directors of Rare Disease Clinical Research Centers, and the Director of the Data and Technology Coordinating Center.

This cooperative agreement is awarded in accordance with RFA-RR-03-008, "Rare Diseases Clinical Research Network."

Funds to support this grant have been provided by The Office of Rare Diseases, Office of the Director, NIH, and the National Center for Research Resources.

RESTRICTION:

NO FUNDS PROVIDED UNDER THIS AWARD MAY BE EXPENDED FOR SUPPORT OF APPROVED PROJECT ACTIVITIES PERFORMED AT THE CONSORTIUM SITE AT THE UNIVERSITY OF SAO PAULO IN SAO PAULO, BRAZIL UNTIL: (1) NCRR HAS RECEIVED WRITTEN NOTIFICATION FROM NIH THAT U.S. DEPARTMENT OF STATE

APPROVAL FOR THIS ACTIVITY HAS BEEN RECEIVED, (2) NCRR HAS NOTIFIED THE GRANTEE INSTITUTION IN WRITING THAT APPROVAL HAS BEEN RECEIVED, AND (3) A REVISED NOTICE OF GRANT AWARD HAS BEEN ISSUED BY NCRR TO THE GRANTEE INSTITUTION REMOVING THIS RESTRICTION FROM THE AWARD.

RESTRICTION:

NO FUNDS PROVIDED UNDER THIS AWARD MAY BE EXPENDED FOR SUPPORT OF PROTOCOLS INVOLVING HUMAN SUBJECTS, EXCEPT FOR EXEMPT RESEARCH AS DESCRIBED IN 45 CFR 46.10(b), UNTIL THE PROTOCOL HAS BEEN REVIEWED AND APPROVED BY THE GRANTEE'S INSTITUTIONAL REVIEW BOARD.

This award includes funds for consortia activity with The University of Texas Southwestern Medical Center, The Columbia University/New York State Psychiatric Institute, The University of Sao Paola, Brazil and Lyon France Site as follows:

	Texas SW Med Ctr	Columbia U/NY State
Personnel	68,373	74,754
Supplies	15,024	1,500
Travel		1,740
Patient Care		
Outpatient	40,000	
Other Expenses	7,500	20,390
<input type="checkbox"/>		

Direct Costs	130,897	98,384
F&A Costs	50,902	51,206
Total Costs	181,799	149,590

	Lyon France	Sao Paola, Brazil
Personnel		36,000
Supplies	40,238	24,000
Travel	5,000	
Patient Care		
Outpatient	45,000	
Other Expenses	5,000	
Direct Costs	95,238	60,000
F&A Costs	4,762	0
Total Costs	100,000	60,000

Consortia are to be established and administered as described in the NIH Grants Policy Statement, March 2001, part II, pages 235-238.

Cooperative Agreement Terms And Conditions Of Award

The following terms and conditions will be incorporated into the award statement and provided to the Principal Investigator as well as the institutional official at the time of award.

These special Terms of Award are in addition to, and not in lieu of, otherwise applicable OMB administrative guidelines, HHS Grant Administration Regulations at 45 CFR part 74 and 92, and other HHS, PHS, and NIH Grant Administration policy statements.

The administrative and funding instrument used for this program is the multiproject cooperative agreement (U54), an "assistance" mechanism rather than an "acquisition" mechanism, in which substantial NIH scientific and/or programmatic involvement with the awardee is anticipated during the performance of the activity. Under the cooperative agreement, the NIH purpose is to support and/or stimulate the recipient's activity by involvement in and otherwise working jointly with the award recipient in a partner role, but it is not to assume direction, prime responsibility, or a dominant role in the activity. Consistent with this concept, the dominant role and prime responsibility for the activity resides with the awardees for the project as a whole, although specific tasks and activities in carrying out the research will

be shared among the awardees and the NIH Research Coordinators.

1. Awardee Rights and Responsibilities

Awardees will have primary responsibility for defining the details of the project within the guidelines of the RFA RR 03-008 and for performing the scientific activity, and agree to accept close coordination, cooperation, and participation of the NIH staff in those aspects of the scientific and technical management of the project described below. Specifically, awardees have primary responsibility as described below.

RDCRC Director and the DTCC Director

The Rare Diseases Clinical Research Center Directors and Data and Technology Coordinating Center Director are the persons responsible for the overall management of their Centers and coordination with the other Centers. The relationship between the Clinical Centers and the Data and Technology Center should be one of equal partners in the Network. Each Center Director must devote at least 20% effort to this program.

Collaboration and Coordination

The collaboration of investigators between Centers is highly encouraged based on shared interests and complementary talents. The planned collaborating sites within the Center must be ongoing and active. Plans for evaluating and removing or replacing non-productive members of a Center consortium must be in place for each Center.

Steering Committee Membership and Meeting Attendance

Each Center Principal Investigator will be designated the Center Director. Each Center Director will be a voting member of the Network Steering Committee and participate in all Committee activities and decisions including, but not limited to, conference calls and special subcommittees as may be necessary. The Steering Committee shall be responsible for determining the frequency of meetings and scheduling the time and location. The Steering committee will establish the procedures for the function of the Centers network, as outlined in section "Steering Committee."

Data Coordination and Management and Sharing

The awardees will have primary rights to all data developed under these awards, subject to Government rights of access consistent with HHS and NIH policies. The DTCC will develop with the input of the Steering Committee a data management system. All Centers will place their data at the DTCC who will also offer analysis expertise for Network investigators. The intention of the NIH is that the data collected within this Network will be become a resource for the Rare Disease Community and will be made available to the scientific community. Criteria and mechanisms for data sharing among investigators within the Network and with the scientific community will be developed by the Steering Committee.

Publication and Presentation of Study Findings

Early publication of major findings is encouraged. Publications and oral presentations of work performed under this agreement will require appropriate acknowledgment of the Rare Disease Clinical Research Network and NIH support. The Steering Committee will establish the procedures and criteria for presentation and publication of data developed within the Centers network.

Federally Mandated Regulatory Requirements

Each institution participating in the Rare Diseases Clinical Research Network is required to meet DHHS regulations for the protection of human subjects and FDA requirements for the conduct of research using investigational agents. At a minimum, these include:

- o methods for assuring that each institution at which Rare Diseases Clinical Research Network investigators are conducting clinical studies has registered with the Office of Human Research Protections (OHRP; <http://ohrp.osophs.dhhs.gov/>) and has a Federalwide Assurance; that study protocols are reviewed and approved by the responsible Institutional Review Board (IRB) prior to patient entry; that active protocols are reviewed at least annually by the IRB, and that amendments are approved by the IRB.

- o methods for assuring or documenting that each patient, or patient's parent/legal guardian, gives fully informed consent to participation in a research protocol prior to the initiation of the clinical study.

2. NIH Staff Responsibilities

One representative from ORD and one representative from the NCRR will be designated to serve as the Program Coordinators for this cooperative agreement. The ORD and NCRR Program Coordinators and one Program Officer from each participating IC will have substantial scientific/programmatic involvement during the conduct of this activity through technical assistance, advice and coordination above and beyond normal program stewardship for grants, as described below.

Steering Committee Membership and Meeting Attendance

The ORD and NCRR Program Coordinators and one Program Officer from each participating IC will serve on the Steering Committee and will participate in all Committee activities, including, but not limited to, meetings, conference calls, subcommittees, and special committees. They will assist in development of operating policies, quality control procedures, and policies that require cooperative action. However, while the ORD and the NCRR Program Coordinators and participating IC

Program Officers will attend Steering Committee Meetings, their cumulative votes may never exceed 40 percent.

Monitoring Performance

The ORD and NCRR Program Coordinators and IC Program Officers will assist the Steering Committee in the development of procedures for monitoring the performance of the clinical studies. This includes participation in periodic on-site monitoring with respect to compliance with protocol specifications, quality control and accuracy of data recording, and accrual. The NIH will also provide assistance to the DTCC in identifying technology resources, provide oversight of activities, including security and privacy issues.

Publication and Presentation of Clinical Studies Findings

The NIH staff may contribute, through review, comment, analysis, and/or co-authorship, to reporting results of the clinical studies and trials/studies to the investigator community and other interested scientific and lay organizations. Co-authorship by the NIH staff will be subject to approval in accordance with the NIH policies regarding staff authorship of publications resulting from extramural awards.

The Government, via the ORD Program Coordinator and the NCRR Program Coordinator, will have access to data generated under this Cooperative Agreement and may periodically review the data and progress reports.

Information obtained from the data may be used by NIH staff for the preparation of internal reports on the activities of the clinical studies. However, awardees will retain custody of and have primary rights to all data developed under these awards.

Program Stewardship

The assigned Program Directors will be responsible for normal programmatic stewardship and monitoring of this award and approval of new pilot studies. The Program Directors may also serve as the NCRR Program Coordinator and the substantively involved IC Program officers. They may receive input and recommendations from other NIH staff in monitoring the awards.

3. Collaborative Responsibilities

All investigators within each Center and the Coordinating Center must be willing to work cooperatively and collaboratively both within their Center consortium and with other Centers. Each Center is expected to send two Center participants to three 2 day meetings in the first year to the Washington, D.C. area and biannually thereafter.

Steering Committee

A Steering Committee will be established to serve as the main governing body of the cooperative network. At a minimum, the Steering Committee will be composed of one representative from each of the RDCR Centers, one representative from the DTCC, the ORD Program Coordinator, the NCRR Program Coordinator, and other participating IC Program Officers. All members are expected to actively participate in all Steering Committee activities. The combined vote of NIH membership may never exceed 40 percent.

The Chairperson of the Steering Committee will be selected by the Steering Committee from among the non-Federal members during one of the early meetings of the Committee to be convened by the NIH Research Coordinators. All major decisions will be determined by the Steering Committee. The Committee will meet at least three times during the first 12 months of the program and at least semi-annually thereafter. As needed, the Steering Committee may establish subcommittees for special purposes. It is expected that most of the work of the Steering Committee will be performed in these subcommittees. All Centers must abide by decisions of the Steering Committee.

The Steering Committee will have responsibility for facilitating the conduct of the clinical studies, promoting trans-Center collaboration,
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establishing and updating the content of the web resource site, and establishing procedures for reporting results of Center studies. The NIH may provide additional funds in future years for new pilot projects. The Steering Committee should develop procedure for reviewing proposals for such projects. The Steering Committee will provide scientific and technical assistance and guidelines with respect to quality control, uniformity of data collection, management of the collective rare diseases database, and data analysis.

4. Arbitration

Any disagreement that may arise on scientific or programmatic matters (within the scope of the award) between award recipients and the NIH may be brought to arbitration. An arbitration panel will be formed to review any scientific or programmatic issue that is significantly restricting progress. This panel will be composed of three members -- one selected by the Steering Committee or by the individual awardee in the event of an individual disagreement, a second member selected by the

NIH, and a third member with expertise in the relevant area and selected by the two prior members. While the decisions of the Arbitration Panel are binding, these special arbitration procedures will in no way affect the awardee's right to appeal an adverse action in accordance with PHS regulations at 42 CFR Part 50, subpart D, and HHS regulations at 45 CFR Part 16.

SECTION V - NCRR CONTACTS:

The Grants Management Specialist is responsible for the negotiation, award and administration of this project and for interpretation of Grants Administration policies and provisions. The Program Official is responsible for the scientific, programmatic and technical aspects of this project. These individuals work together in overall project administration. Prior approval requests (countersigned by the PI & authorized business official) should be submitted in writing to the Grants Management Specialist. Requests may be made via e-mail provided they are routed through these same officials.

The NCRR WWW home page is at <http://www.ncrr.nih.gov/>

Elaine S. Collier, Program Official
 Judith Musgrave, Grants Specialist
 Phone: (301) 435-0841 Email: musgravj@mail.nih.gov Fax: (301) 480-3777

SPREADSHEET
 GRANT NUMBER: 7 U54 RR019484-02 (Revised)

P.I.: NEW, MARIA I.
 INSTITUTION: MOUNT SINAI SCHOOL OF MEDICINE OF NYU

	YEAR 02	YEAR 03	YEAR 04	YEAR 05	YEAR 06
	=====	=====	=====	=====	=====
Salaries and Wages	38,066	228,393	241,588	246,281	253,672
Fringe Benefits	9,706	58,241	61,605	62,801	64,686
Personnel Costs	47,772	286,634	303,193	309,082	318,358
Equipment	6,000				
Supplies	52,500	12,131	13,260	13,658	14,069
Travel Costs	16,000	16,060	14,000	14,000	14,000
Patient Care (Outpatient)	40,000	40,000	40,000	40,000	40,000
Other Costs	74,431	23,500	89,293	33,929	34,947
□ Consortium/Contractual Cost	491,389	509,876	405,019	406,705	448,698
TOTAL FEDERAL DC	728,092	888,201	864,765	817,374	870,072
TOTAL FEDERAL F&A	202,039	235,136	291,724	257,616	265,055
TOTAL COST	930,131	1,123,337	1,156,489	1,074,990	1,135,127
□					

	YEAR 02	YEAR 03	YEAR 04	YEAR 05	YEAR 06
	=====	=====	=====	=====	=====
F&A Cost Rate 1	69.50%	69.50%	69.50%	69.50%	69.50%
F&A Cost Base 1	290,703	338,325	419,747	370,670	381,374
F&A Costs 1	202,039	235,136	291,724	257,616	265,055

.....END OF NGA.....

***** NOTICE OF GRANT AWARD *****
 Issue Date:07/27/2004

Department of Health and Human Services
 National Institutes of Health

NATIONAL CENTER FOR RESEARCH RESOURCES

Grant Number: 7 U54 RR019484-02
 Principal Investigator: NEW, MARIA I. MD
 Project Title: Natural History of Rare Genetic Steroid Disorders

MS JESSICA MOISE
 DIRECTOR
 GRANTS AND CONTRACTS OFFICE
 MOUNT SINAI SCHOOL OF MEDICINE
 NEW YORK, NY 100296574
 NEW YORK, NY 100296574
 UNITED STATES
 Award e-mailed to: Grants@mssm.edu

Budget Period: 06/01/2004 - 07/31/2004
 Project Period: 09/30/2003 - 07/31/2008

Dear Business Official:

The National Institutes of Health hereby awards a grant in the amount of \$930,131(see 'Award Calculation' in Section I) to MOUNT SINAI SCHOOL OF MEDICINE OF NYU in support of the above referenced project. This award is pursuant to the authority of and is subject to terms and conditions referenced below.

Acceptance of this award including the Terms and Conditions is acknowledged by the grantee when funds are drawn down or otherwise obtained from the grant payment system.

Award recipients are responsible for reporting inventions derived or reduced to practice in the performance of work under this grant. Rights to inventions vest with the grantee organization provided certain requirements are met and there is acknowledgement of NIH support. In addition, recipients must ensure that patent and license activities are consistent with their responsibility to make unique research resources developed under this award available to the scientific community, in accordance with NIH policy. For additional information, please visit <http://www.iedison.gov>.

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Sincerely yours,

Mary Niemiec
 Grants Management Officer
 NATIONAL CENTER FOR RESEARCH RESOURCES

See additional information below

SECTION I - AWARD DATA - 7 U54 RR019484-02

AWARD CALCULATION (U.S. Dollars):

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TOTAL FEDERAL AWARD AMOUNT	\$930,131

Recommended future year total cost support, subject to the availability of funds and satisfactory progress of the project, is as follows.

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04	\$1,156,489
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FISCAL INFORMATION:

CFDA 93.389
 Number:
 EIN: XXXXXXXXXX
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IC/	CAN	/	FY2003	/	FY2004	/	FY2005	/	FY2006	/	FY2007
OD/8421751/			930,130/		1,123,336/		1,156,488/		1,074,989/		1,135,126
RR/8464629/			1/		1/		1/		1/		1

NIH ADMINISTRATIVE DATA:

PCC: CRT65 / OC: 41.4P /Processed: NIEMIECM 040723 0659

SECTION II - PAYMENT/HOTLINE INFORMATION - 7 U54 RR019484-02

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SECTION III - TERMS AND CONDITIONS - 7 U54 RR019484-02

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 - b. The restrictions on the expenditure of federal funds in appropriations acts, to the extent those restrictions are pertinent to the award.
 - c. 45 CFR Part 74 or 45 CFR Part 92 as applicable.
 - d. The NIH Grants Policy Statement, including addenda in effect as of the beginning date of the budget period.
 - e. This award notice, INCLUDING THE TERMS AND CONDITIONS CITED BELOW.
-

(see NIH Home Page at
<http://grants.nih.gov/grants/policy/awardconditions.htm> for certain references cited above.)

Carry over of an unobligated balance into the next budget period requires Grants Management Officer prior approval.

Treatment of Program Income:
Other Research (Add/Deduct Option)

SECTION IV - NCRR ADDITIONAL TERMS AND CONDITIONS - 7 U54 RR019484-02

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This cooperative agreement is awarded in accordance with RFA-RR-03-008, "Rare Diseases Clinical Research Network."

Funds to support this grant have been provided by The Office of Rare Diseases, Office of the Director, NIH, and the National Center for Research Resources.

RESTRICTION:

NO FUNDS PROVIDED UNDER THIS AWARD MAY BE EXPENDED FOR SUPPORT OF APPROVED PROJECT ACTIVITIES PERFORMED AT THE CONSORTIUM SITE AT THE UNIVERSITY OF SAO PAULO IN SAO PAULO, BRAZIL UNTIL: (1) NCRR HAS RECEIVED WRITTEN NOTIFICATION FROM NIH THAT U.S. DEPARTMENT OF STATE APPROVAL FOR THIS ACTIVITY HAS BEEN RECEIVED, (2) NCRR HAS NOTIFIED THE GRANTEE INSTITUTION IN WRITING THAT APPROVAL HAS BEEN RECEIVED, AND (3) A REVISED NOTICE OF GRANT AWARD HAS BEEN ISSUED BY NCRR TO THE GRANTEE INSTITUTION REMOVING THIS RESTRICTION FROM THE AWARD.

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Personnel		36,000
Supplies	40,238	24,000
Travel	5,000	
Patient Care		
Outpatient	45,000	
Other Expenses	5,000	
□		

Direct Costs	95,238	60,000
F&A Costs	4,762	0
Total Costs	100,000	60,000

Consortia are to be established and administered as described in the NIH Grants Policy Statement, March 2001, part II, pages 235-238.

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One representative from ORD and one representative from the NCRR will be designated to serve as the Program Coordinators for this cooperative agreement. The ORD and NCRR Program Coordinators and one Program Officer from each participating IC will have substantial scientific/programmatic involvement during the conduct of this activity through technical assistance, advice and coordination above and beyond normal program stewardship for grants, as described below.

Steering Committee Membership and Meeting Attendance

The ORD and NCRR Program Coordinators and one Program Officer from each participating IC will serve on the Steering Committee and will participate in all Committee activities, including, but not limited to, meetings, conference calls, subcommittees, and special committees. They will assist in development of operating policies, quality control procedures, and policies that require cooperative action. However, while the ORD and the NCRR Program Coordinators and participating IC Program Officers will attend Steering Committee Meetings, their cumulative votes may never exceed 40 percent.

Monitoring Performance

The ORD and NCRR Program Coordinators and IC Program Officers will assist the Steering Committee in the development of procedures for monitoring the performance of the clinical studies. This includes participation in periodic on-site monitoring with respect to compliance with protocol specifications, quality control and accuracy of data recording, and accrual. The NIH will also provide assistance to the

DTCC in identifying technology resources, provide oversight of activities, including security and privacy issues.

Publication and Presentation of Clinical Studies Findings

The NIH staff may contribute, through review, comment, analysis, and/or co-authorship, to reporting results of the clinical studies and trials/studies to the investigator community and other interested scientific and lay organizations. Co-authorship by the NIH staff will be subject to approval in accordance with the NIH policies regarding staff authorship of publications resulting from extramural awards.

The Government, via the ORD Program Coordinator and the NCRR Program Coordinator, will have access to data generated under this Cooperative Agreement and may periodically review the data and progress reports. Information obtained from the data may be used by NIH staff for the preparation of internal reports on the activities of the clinical studies. However, awardees will retain custody of and have primary rights to all data developed under these awards.

Program Stewardship

The assigned Program Directors will be responsible for normal programmatic stewardship and monitoring of this award and approval of

new pilot studies. The Program Directors may also serve as the NCRR Program Coordinator and the substantively involved IC Program officers. They may receive input and recommendations from other NIH staff in monitoring the awards.

3. Collaborative Responsibilities

All investigators within each Center and the Coordinating Center must be willing to work cooperatively and collaboratively both within their Center consortium and with other Centers. Each Center is expected to send two Center participants to three 2 day meetings in the first year to the Washington, D.C. area and biannually thereafter.

Steering Committee

A Steering Committee will be established to serve as the main governing body of the cooperative network. At a minimum, the Steering Committee will be composed of one representative from each of the RDCR Centers, one representative from the DTCC, the ORD Program Coordinator, the NCRR Program Coordinator, and other participating IC Program Officers. All members are expected to actively participate in all Steering Committee activities. The combined vote of NIH membership may never exceed 40 percent.

The Chairperson of the Steering Committee will be selected by the Steering Committee from among the non-Federal members during one of the early meetings of the Committee to be convened by the NIH Research Coordinators. All major decisions will be determined by the Steering Committee. The Committee will meet at least three times during the first 12 months of the program and at least semi-annually thereafter. As needed, the Steering Committee may establish subcommittees for special purposes. It is expected that most of the work of the Steering Committee will be performed in these subcommittees. All Centers must abide by decisions of the Steering Committee.

The Steering Committee will have responsibility for facilitating the conduct of the clinical studies, promoting trans-Center collaboration, establishing and updating the content of the web resource site, and establishing procedures for reporting results of Center studies. The NIH may provide additional funds in future years for new pilot projects. The Steering Committee should develop procedure for reviewing proposals for such projects. The Steering Committee will provide scientific and technical assistance and guidelines with respect to quality control, uniformity of data collection, management of the collective rare diseases database, and data analysis.

4. Arbitration

Any disagreement that may arise on scientific or programmatic matters (within the scope of the award) between award recipients and the NIH may be brought to arbitration. An arbitration panel will be formed to review any scientific or programmatic issue that is significantly restricting progress. This panel will be composed of three members -- one selected by the Steering Committee or by the individual awardee in the event of an individual disagreement, a second member selected by the NIH, and a third member with expertise in the relevant area and selected by the two prior members. While the decisions of the Arbitration Panel are binding, these special arbitration procedures will in no way affect the awardee's right to appeal an adverse action in accordance with PHS regulations at 42 CFR Part 50, subpart D, and HHS regulations at 45 CFR Part 16.

SECTION V - NCRR CONTACTS:

The Grants Management Specialist is responsible for the negotiation,

award and administration of this project and for interpretation of Grants Administration policies and provisions. The Program Official is responsible for the scientific, programmatic and technical aspects of this project. These individuals work together in overall project administration. Prior approval requests (countersigned by the PI & authorized business official) should be submitted in writing to the Grants Management Specialist. Requests may be made via e-mail provided they are routed through these same officials.

The NCRR WWW home page is at <http://www.ncrr.nih.gov/>

Elaine S. Collier, Program Official
 Judith Musgrave, Grants Specialist
 Phone: (301) 435-0841 Email: musgravj@mail.nih.gov Fax: (301) 480-3777

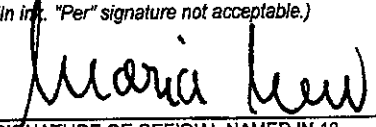
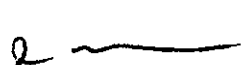
SPREADSHEET
 GRANT NUMBER: 7 U54 RR019484-02

P.I.: NEW, MARIA I.
 INSTITUTION: MOUNT SINAI SCHOOL OF MEDICINE OF NYU

	YEAR 02	YEAR 03	YEAR 04	YEAR 05	YEAR 06
	=====	=====	=====	=====	=====
Salaries and Wages	38,066	228,393	241,588	246,281	253,672
Fringe Benefits	9,706	58,241	61,605	62,801	64,686
Personnel Costs	47,772	286,634	303,193	309,082	318,358
Equipment	6,000				
Supplies	52,500	12,131	13,260	13,658	14,069
Travel Costs	16,000	16,060	14,000	14,000	14,000
Patient Care (Outpatient)	40,000	40,000	40,000	40,000	40,000
Other Costs	74,431	23,500	89,293	33,929	34,947
Consortium/Contractual Cost	491,389	509,876	405,019	406,705	448,698
TOTAL FEDERAL DC	728,092	888,201	864,765	817,374	870,072
TOTAL FEDERAL F&A	202,039	235,136	291,724	257,616	265,055
TOTAL COST	930,131	1,123,337	1,156,489	1,074,990	1,135,127

	YEAR 02	YEAR 03	YEAR 04	YEAR 05	YEAR 06
	=====	=====	=====	=====	=====
□F&A Cost Rate 1	69.50%	69.50%	69.50%	69.50%	69.50%
F&A Cost Base 1	290,703	338,325	419,747	370,670	381,374
F&A Costs 1	202,039	235,136	291,724	257,616	265,055

.....END OF NGA.....

Department of Health and Human Services Public Health Service Grant Application Follow instructions carefully. Do not exceed 56-character length restrictions, including spaces.		LEAVE BLANK-FOR PHS USE ONLY. Type <u>7</u> Activity <u>154</u> Number <u>RRO19484-03</u> Review Group _____ Formerly _____ Council/Board (Month, Year) _____ Date Received _____	
1. TITLE OF PROJECT The Natural History of Rare Genetic Steroid Disorders			
2. RESPONSE TO SPECIFIC REQUEST FOR APPLICATIONS OR PROGRAM ANNOUNCEMENT OR SOLICITATION <input checked="" type="checkbox"/> NO <input type="checkbox"/> YES (If "Yes," state number and title) Number: _____ Title: _____			
3. PRINCIPAL INVESTIGATOR/PROGRAM DIRECTOR 3a. NAME (Last, first, middle) New, Maria I.		New Investigator <input checked="" type="checkbox"/> No <input type="checkbox"/> Yes 3b. DEGREE(S) MD	
3c. POSITION TITLE Professor		3d. MAILING ADDRESS (Street, city, state, zip code) Mount Sinai School of Medicine One Gustave L. Levy Place New York, NY 10029-6574	
3e. DEPARTMENT, SERVICE, LABORATORY, OR EQUIVALENT Pediatrics		E-MAIL ADDRESS: maria.new@mssm.edu	
3f. MAJOR SUBDIVISION School of Medicine		3g. TELEPHONE AND FAX (Area code, number and extension) TEL: (212) 241-8210 FAX: (212) 876-4395	
4. HUMAN SUBJECTS RESEARCH <input type="checkbox"/> No <input checked="" type="checkbox"/> Yes 4a. Research Exempt <input checked="" type="checkbox"/> No <input type="checkbox"/> Yes If "Yes," Exemption no. _____ 4b. Human Subjects Assurance No. 00005656 4c. NIH-defined Phase III Clinical Trial <input checked="" type="checkbox"/> No <input type="checkbox"/> Yes		5. VERTEBRATE ANIMALS <input checked="" type="checkbox"/> No <input type="checkbox"/> Yes 5a. If "Yes," IACUC approval Date _____ 5b. Animal welfare assurance no A3111-01	
6. DATES OF PROPOSED PERIOD OF SUPPORT (month, day, year-MM/DD/YY) From 06/01/04 Through 07/31/08		7. COSTS REQUESTED FOR INITIAL BUDGET PERIOD 7a. Direct Costs (\$) 758,116. 7b. Total Costs (\$) 930,130.	
		8. COSTS REQUESTED FOR PROPOSED PERIOD OF SUPPORT 8a. Direct Costs (\$) 4,465,282. 8b. Total Costs (\$) 5,614,913.	
9. APPLICANT ORGANIZATION Name Mount Sinai School of Medicine Address One Gustave L. Levy Place, Box 1075 New York, NY 10029-6574		10. TYPE OF ORGANIZATION Public: <input type="checkbox"/> Federal <input type="checkbox"/> State <input type="checkbox"/> Local Private: <input checked="" type="checkbox"/> Private Nonprofit Forprofit: <input type="checkbox"/> General <input type="checkbox"/> Small Business <input type="checkbox"/> Woman-owned: <input type="checkbox"/> Socially and Economically Disadvantaged	
Institutional Profile Number (if known) _____		11. ENTITY IDENTIFICATION NUMBER DUNS NO. (if available) 78861598 Congressional District 14	
12. ADMINISTRATIVE OFFICIAL TO BE NOTIFIED IF AWARD IS MADE Name Ms. Jessica Moise Title Director Address Grants and Contracts Office Mount Sinai School of Medicine One Gustave L. Levy Place, Box 1075 New York, NY 10029-6574 Tel (212) 659-8970 FAX (212) 876-6789 E-Mail grants@mssm.edu		13. OFFICIAL SIGNING FOR APPLICANT ORGANIZATION Name Ravi Ivengar, PhD Title Dean for Research Address Grants and Contracts Office Mount Sinai School of Medicine One Gustave L. Levy Place, Box 1075 New York, NY 10029-6574 Tel (212) 659-8970 FAX (212) 876-6789 E-Mail grants@mssm.edu	
14. PRINCIPAL INVESTIGATOR/PROGRAM DIRECTOR ASSURANCE: I certify that the statements herein are true, complete and accurate to the best of my knowledge. I am aware that any false, fictitious, or fraudulent statements or claims may subject me to criminal, civil, or administrative penalties. I agree to accept responsibility for the scientific conduct of the project and to provide the required progress reports if a grant is awarded as a result of this application.		SIGNATURE OF PI/PD NAMED IN 3a. (In ink. "Per" signature not acceptable.) 	DATE 05/26/04
15. APPLICANT ORGANIZATION CERTIFICATION AND ACCEPTANCE: I certify that the statements herein are true, complete and accurate to the best of my knowledge, and accept the obligation to comply with Public Health Service terms and conditions if a grant is awarded as a result of this application. I am aware that any false, fictitious, or fraudulent statements or claims may subject me to criminal, civil, or administrative penalties.		SIGNATURE OF OFFICIAL NAMED IN 13. (In ink. "Per" signature not acceptable.) 	DATE 05/26/04

DESCRIPTION: State the application's broad, long-term objectives and specific aims, making reference to the health relatedness of the project. Describe concisely the research design and methods for achieving these goals. Avoid summaries of past accomplishments and the use of the first person. This abstract is meant to serve as a succinct and accurate description of the proposed work when separated from the application. If the application is funded, this description, as is, will become public information. Therefore, do not include proprietary/confidential information. **DO NOT EXCEED THE SPACE PROVIDED.**

A consortium of investigators, institutions, and patient support groups will constitute a Rare Disease Clinical Research Network focused on a diverse group of disorders characterized by defects in steroidogenesis. **We will study the longitudinal history of these rare disorders and determine the outcome of treatment on height, fertility and gender.** Long-standing informal collaboration between investigators at Mount Sinai School of Medicine, Rockefeller University, Columbia University, the University of Texas Southwestern Medical Center, the University of Quebec, Hopital Debrosses (Lyons), and the Hospital das Clinicas da FMUSP (Sao Paulo) will facilitate the creation of a productive cooperative research network that draws on the extensive experience of each investigator. Clinical Research Centers at Mount Sinai, Rockefeller, and the University of Texas Southwestern Medical Center will participate. Each investigator in the consortium has followed a large group of patients with a specific genetic defect affecting steroid synthesis over many years, encompassing the natural history of these diseases from prenatal life to death. Creation of a storage and management database will constitute a scaffold for ongoing research, enabling the preservation and use of this large body of clinical data assembled by experts in each disorder. Moreover, design of templates for a standardized clinical description of these disorders will permit prospective studies which can offer open enrollment to affected individuals or individuals at risk. Our research group includes the investigators who have identified the molecular genetic defect for each disorder, where known, and who maintain laboratories dedicated to the identification of new mutations. The combination of clinical and molecular genetic information will raise the standard of medical care and may permit development of novel treatments based on detailed knowledge of the natural history and molecular genetic basis of these disorders. Important elements of our plan are (1) to establish the clinical research network which pools data from our sites in cooperation with the DTCC and analyzes this data, (2) to educate young investigators in the management and clinical research of steroid disorders, and (3) to strengthen our connections with patient support groups to enable individuals affected or at risk to have new kinds of input and access to optimal medical care.

PERFORMANCE SITE(S) (organization, city, state)

Mount Sinai School of Medicine, New York NY

Rockefeller University, New York NY

Laval University, Quebec, Canada

University of Texas Southwestern Medical Center, Dallas TX

Hopital Debrosses Lyon, France

Hospital das Clinicas da FMUSP, Sao Paulo, Brazil

Columbia College of Physicians and Surgeons/New York State Psychiatric Institute, New York NY

KEY PERSONNEL. See instructions. Use continuation pages as needed to provide the required information in the format shown below. Start with Principal Investigator. List all other key personnel in alphabetical order, last name first.

Name	Organization	Role on Project
New Maria	Professor, Mt. Sinai, NY NY	Principal investigator
Arnhold, Ivo	Professor, University of Sao Paulo	Site PI: Sao Paulo
Auchus, Richard	Asst. Prof., U Texas SW Med Ctr	Co-investigator
Baker, Susan	Asst. Prof., Mt. Sinai, NY NY	Co-investigator
Correa, Raffaella	Physician, University of Sao Paulo	Co-investigator
Costa, Elaine	Physician, University of Sao Paulo	Co-investigator
Dolezal, Curtis	Assistant Prof. Columbia	Co-investigator
Domenice, Sorahia	Physician, University of Sao Paulo	Co-investigator
Forest, Maguelone	Professor Emeritus, Lyon, France	Co-investigator
Harbison, Madeleine	Asst. Prof., Mt. Sinai, NY NY	Co-investigator
Inacio, Marlene	Psychologist, University of Sao Paulo	Co-investigator
Macapagal, Maria Cristina	Asst. Prof., Mt. Sinai NY NY	Co-investigator

Disclosure Permission Statement Applicable to SBIR/STTR Only See instructions Yes No

New Maria I.

Martin, Regina	Physician, University of Sao Paolo	Co-investigator
Marui, Suemi	Physician, University of Sao Paolo	Co-investigator
Mendonca, Berenice	Assoc. Prof, University of Sao Paolo	Program Dir., Sao Paolo
Meyer-Bahlburg, Heino	Professor, Columbia University, NY NY	Site PI: Gender Outcome
Morel, Yves	Professor, University of Lyons	Site PI: Lyon
Obeid, Jihad	Asst. Prof, Weill Medical College	Co-investigator
Russell, David	Professor, U Texas SW Med Ctr	Co-investigator
Simard, Jacques	Professor, U Laval, Quebec	Co-investigator
Verduguez, Elisa	Psychologist, University of Sao Paolo	Co-investigator
Wilson, Jean	Professor, U Texas SW Med Ctr	Site PI: Dallas
Wilson, Robert	Assoc. Prof., Mt. Sinai, NY NY	Co-investigator
<hr/>		
Chervenak, Frank	Chairman OB/GYN Department; Given Foundation Professor, Weill Cornell, NY NY	Consultant: Imaging
Pass, Kenneth	Chief, Laboratory of Genetic Services, NY State Newborn Screening and Genetic Services, Albany NY	Consultant: Recruitment
Polaneczy, Margaret	Assoc. Prof. OB/GYN, Weill Cornell, NY NY	Consultant: OB/GYN
Poppas, Dix	Richard Rogers Family Associate Professor of Pediatric Urology; Chief, Pediatric Urology and Reconstructive Surgery, Weill Cornell, NY NY	Consultant: Genital surgery
Rosenwaks, Zev	Director of Institute for Reproductive Medicine; Revlon Distinguished Professor, Weill Cornell, NY NY	Consultant: IVF
Schlegel, Peter	Acting Chairman of Urology; Assoc. Professor, Weill Cornell, NY NY	Consultant: Male fertility
Vaughan, Darracott	James Colt Professor of Urology, Weill Cornell, NY NY	Consultant: Male fertility
<hr/>		
Leight, Kelly	Director, CARES Foundation	Patient Support Group