

Rett Syndrome Research Trust, Inc.

Financial Statements

December 31, 2015



Rett Syndrome Research Trust, Inc.
December 31, 2015

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Report of Independent Auditor

To the Board of Trustees
Rett Syndrome Research Trust, Inc.

We have audited the accompanying financial statements of Rett Syndrome Research Trust, Inc. (a nonprofit organization) which comprise the statements of financial position as of December 31, 2015 and 2014 and the related statements of activities, cash flows and functional expenses for the years then ended, and the related notes to the financial statements.

Management's Responsibility for the Financial Statements

Management is responsible for the preparation and fair presentation of these financial statements in accordance with accounting principles generally accepted in the United States of America; this includes the design, implementation, and maintenance of internal control relevant to the preparation and fair presentation of financial statements that are free from material misstatement, whether due to fraud or error.

Auditor's Responsibility

Our responsibility is to express an opinion on these financial statements based on our audits. We conducted our audits in accordance with auditing standards generally accepted in the United States of America. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free from material misstatement.

An audit involves performing procedures to obtain audit evidence about the amounts and disclosures in the financial statements. The procedures selected depend on the auditor's judgment including the assessment of the risks of material misstatement of the financial statements, whether due to fraud or error. In making those risk assessments, the auditor considers internal control relevant to the entity's preparation and fair presentation of the financial statements in order to design audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the entity's internal control. Accordingly, we express no such opinion. An audit also includes evaluating the appropriateness of accounting policies used and the reasonableness of significant accounting estimates made by management, as well as evaluating the overall presentation of the financial statements.

We believe that the audit evidence we have obtained is sufficient and appropriate to provide a basis for our audit opinion.

Other Matter

Our audits were conducted for the purpose of forming an opinion on the financial statements as a whole. The Supplemental Statements of Functional Expense Allocation and Research Funded Expense Graphs, and the Research Awards and Grant Commitments and Payments Schedule are presented for purposes of additional analysis and are not a required part of the basic financial statements. Such information is the responsibility of management and was derived from and relates directly to the underlying accounting and other records used to prepare the financial statements. The information has been subjected to the auditing procedures applied in the audit of the financial statements and certain additional procedures, including comparing and reconciling such information directly to the underlying accounting and other records used to prepare the financial statements or to the financial statements themselves, and other additional procedures in accordance with auditing standards generally accepted in the United States of America. In our opinion, the information is fairly stated in all material respects in relation to the financial statements as a whole.

Opinion

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of Rett Syndrome Research Trust, Inc. as of December 31, 2015 and 2014, and the changes in its net assets and its cash flows for the years then ended in accordance with accounting principles generally accepted in the United States of America.

Knight Rolleri Sheppard CPAS, LLP

Knight Rolleri Sheppard CPAS, LLP
Fairfield, Connecticut
April 18, 2016

Rett Syndrome Research Trust, Inc.
Statements of Financial Position
December 31, 2015 and 2014

| | <u>2015</u> | <u>2014</u> |
|--|--------------|--------------|
| <u>ASSETS</u> | | |
| Current assets | | |
| Cash and cash equivalents | \$ 4,330,089 | \$ 5,002,498 |
| Promises to give | 725,738 | 564,549 |
| Investments | 14,002 | 20 |
| Total current assets | 5,069,829 | 5,567,067 |
| Property and equipment, net of accumulated depreciation of \$2,172 and \$1,086 in 2015 and 2014, respectively | | |
| | 1,086 | 2,171 |
| Other assets | | |
| Promises to give | - | 680,880 |
| Total other assets | - | 680,880 |
| Total assets | \$ 5,070,915 | \$ 6,250,118 |
| <u>LIABILITIES AND NET ASSETS</u> | | |
| Current liabilities | | |
| Accounts payable | \$ 14,821 | \$ 2,500 |
| Grants payable | 6,639,332 | 5,537,722 |
| Accrued expenses | 8,059 | 15,548 |
| Total current liabilities | 6,662,212 | 5,555,770 |
| Other liabilities | | |
| Grants payable | 5,465,104 | 3,888,854 |
| Total liabilities | 12,127,316 | 9,444,624 |
| Net assets | | |
| For mission purposes | (7,057,487) | (3,196,677) |
| Property and equipment | 1,086 | 2,171 |
| Total net assets (deficit) | (7,056,401) | (3,194,506) |
| Total liabilities and net assets | \$ 5,070,915 | \$ 6,250,118 |

See report of independent auditor and notes to financial statements.

Rett Syndrome Research Trust, Inc.
Statements of Activities
For the Years Ended December 31, 2015 and 2014

| | <u>2015</u> | <u>2014</u> |
|---|------------------------------|------------------------------|
| Public support and revenues | | |
| Contributions | \$ 5,818,413 | \$ 5,629,477 |
| The Eva Fini Fund | 172,200 | 140,406 |
| Donated services and rent | 7,480 | 8,990 |
| Interest income | 13,509 | 14,729 |
| Realized gain (loss) from sales of donated securities | (59) | 1 |
| Total support and revenue | <u>6,011,543</u> | <u>5,793,603</u> |
| Expenses | | |
| Program services | 9,259,015 | 6,121,490 |
| Supporting services: | | |
| Management and general | 150,292 | 93,634 |
| Fundraising | 464,131 | 358,607 |
| Total expenses | <u>9,873,438</u> | <u>6,573,731</u> |
| Decrease in net assets from operations | (3,861,895) | (780,128) |
| Net assets (deficit) - beginning of year | <u>(3,194,506)</u> | <u>(2,414,378)</u> |
| Net assets (deficit) - end of year | <u><u>\$ (7,056,401)</u></u> | <u><u>\$ (3,194,506)</u></u> |

See report of independent auditor and notes to financial statements.

Rett Syndrome Research Trust, Inc.
Statements of Cash Flows
For the Years Ended December 31, 2015 and 2014

| | <u>2015</u> | <u>2014</u> |
|---|---------------------|---------------------|
| Cash flows from (used in) operating activities | | |
| Decrease in net assets | \$ (3,861,895) | \$ (780,128) |
| Adjustments to reconcile increase (decrease) in net assets to cash from (used in) operating activities | | |
| Amortization | 1,086 | 1,086 |
| (Increase) decrease in promises to give | 519,691 | (165,761) |
| Increase in investments from donor donations held for sale | (13,982) | - |
| Increase (decrease) in accounts payable | 12,321 | (4,184) |
| Decrease in accrued expenses | (7,489) | (6) |
| Increase in grants payable | 2,677,859 | 1,260,372 |
| Net cash provided from (used by) operating activities | <u>(672,409)</u> | <u>311,379</u> |
| Cash flows from investing activities | | |
| Investment in office equipment | <u>-</u> | <u>(3,257)</u> |
| Net cash used in investing activities | - | (3,257) |
| Increase (decrease) in cash | <u>(672,409)</u> | <u>308,122</u> |
| Cash and cash equivalents - beginning of year | <u>5,002,498</u> | <u>4,694,376</u> |
| Cash and cash equivalents - end of year | <u>\$ 4,330,089</u> | <u>\$ 5,002,498</u> |

See report of independent auditor and notes to financial statements.

Rett Syndrome Research Trust, Inc.
Statement of Functional Expenses
For the Year Ended December 31, 2015

| | <u>Program</u> | <u>Supporting Services</u> | | <u>Total Expenses</u> |
|-------------------------------------|---------------------|-------------------------------|--------------------|-----------------------|
| | | <u>Management and General</u> | <u>Fundraising</u> | |
| Awards and grants | \$ 8,782,977 | \$ - | \$ - | \$ 8,782,977 |
| Bank and credit card fees | 9,598 | 5,932 | 12,155 | 27,685 |
| Bookkeeping (**) | 1,119 | 1,119 | 1,119 | 3,357 |
| Depreciation | 1,056 | 4 | 26 | 1,086 |
| Employee benefits | 9,186 | 5,677 | 11,632 | 26,495 |
| Fundraising | - | - | 214,304 | 214,304 |
| Insurance | 1,456 | 1,455 | 1,455 | 4,366 |
| Miscellaneous | 1,280 | 791 | 13,676 | 15,747 |
| Office and supplies | 1,081 | 1,025 | 1,369 | 3,475 |
| Payroll taxes | 9,879 | 6,106 | 12,511 | 28,496 |
| Postage and shipping | 3,068 | 1,896 | 3,886 | 8,850 |
| Printing and publication | 7,484 | 27 | 187 | 7,698 |
| Professional fees (*) | 106,025 | 32,667 | - | 138,692 |
| Professional fees - patent research | 11,197 | - | - | 11,197 |
| Rent (**) | 3,000 | - | - | 3,000 |
| Salaries | 150,107 | 92,773 | 190,093 | 432,973 |
| Scientific meetings | 88,417 | - | - | 88,417 |
| Telephone | 1,314 | 812 | 1,664 | 3,790 |
| Travel - scientific meetings | 58,113 | - | - | 58,113 |
| Website and hosting | 10,495 | - | - | 10,495 |
| | <u>\$ 9,259,015</u> | <u>\$ 150,292</u> | <u>\$ 464,131</u> | <u>\$ 9,873,438</u> |

(*) Includes \$5,000 of donated services.

(**) Includes donated services and rent.

See report of independent auditor and notes to financial statements.

Rett Syndrome Research Trust, Inc.
Statement of Functional Expenses
For the Year Ended December 31, 2014

| | <u>Program</u> | <u>Supporting Services</u> | | <u>Total Expenses</u> |
|-------------------------------------|---------------------|-------------------------------|--------------------|-----------------------|
| | | <u>Management and General</u> | <u>Fundraising</u> | |
| Amortization | \$ 147 | \$ - | \$ - | \$ 147 |
| Awards and grants | 5,861,289 | - | - | 5,861,289 |
| Bank and credit card fees | 3,414 | 2,006 | 4,980 | 10,400 |
| Bookkeeping (**) | 1,083 | 1,083 | 1,083 | 3,249 |
| Depreciation | 1,055 | 2 | 29 | 1,086 |
| Employee benefits | 3,675 | 2,159 | 5,360 | 11,194 |
| Fundraising | - | - | 161,268 | 161,268 |
| Graphic design | - | - | - | - |
| Insurance | 1,541 | 1,541 | 1,541 | 4,623 |
| Miscellaneous | 16 | 10 | 2,787 | 2,813 |
| Office and supplies | 78 | 3,904 | 113 | 4,095 |
| Payroll taxes | 7,383 | 4,337 | 10,768 | 22,488 |
| Postage and shipping | 1,080 | 634 | 1,575 | 3,289 |
| Printing and publication | 2,564 | 6 | 71 | 2,641 |
| Professional fees (*) | - | 9,872 | - | 9,872 |
| Professional fees - patent research | 29,996 | - | - | 29,996 |
| Rent (**) | 3,000 | - | - | 3,000 |
| Salaries | 114,844 | 67,459 | 167,490 | 349,793 |
| Scientific communications | - | - | - | - |
| Scientific meetings | 88,230 | - | - | 88,230 |
| Telephone | 1,058 | 621 | 1,542 | 3,221 |
| Travel - scientific meetings | 694 | - | - | 694 |
| Website and hosting | 343 | - | - | 343 |
| | <u>\$ 6,121,490</u> | <u>\$ 93,634</u> | <u>\$ 358,607</u> | <u>\$ 6,573,731</u> |

(*) Includes \$5,000 of donated services.

(**) Includes donated services and rent.

See report of independent auditor and notes to financial statements.

Rett Syndrome Research Trust, Inc.
Notes to Financial Statements
December 31, 2015
(See Report of Independent Auditor)

NOTE 1 – Organization

Rett Syndrome Research Trust, Inc. (the “Organization” and “RSRT”) is a not-for-profit organization formed under Internal Revenue Code 501(c)(3). The organization is focused on the development of treatments and cures for Rett Syndrome and related *MECP2* disorders. The Organization funds research projects and organizes and sponsors scientific workshops. Equally important, RSRT brings to the scientific community a deep knowledge of the disease and access to intellectual capital. It provides a comprehensive view of the field and a focus on translational efforts.

NOTE 2 – Significant Accounting Policies

Financial Statement Presentation

The Organization prepares its financial statements using guidelines established under Statement on Financial Accounting Standard Codification 958. This Standard requires that a non-profit organization provide information regarding the net assets of the organization based on the existence or absence of donor-imposed restrictions. The types of restrictions are described as follows:

Temporarily restricted net assets -The part of net assets of a not-for-profit organization resulting from contributions and other inflows of assets whose use by the organization is limited by donor – imposed stipulations that either expire by passage of time or can be fulfilled by actions of the organization pursuant to those stipulations.

Permanently restricted net assets - The part of net assets of a not-for-profit organization resulting from contributions and other inflows of assets whose use by the organization is limited by donor – imposed stipulations that neither expire by passage of time nor can be fulfilled or otherwise removed by actions of the organization pursuant to those stipulations.

Unrestricted net assets- The part of net assets of a not-for-profit organization that is neither permanently restricted nor temporarily restricted by donor- imposed stipulations.

Contributions

The Organization reports gifts of cash and other assets as restricted support if they are received with donor stipulations that limit the use of the donated assets. When a donor restriction expires, that is, when a stipulated time restriction ends or purpose restriction is accomplished, temporarily restricted net assets are reclassified to unrestricted net assets and reported in the statement of activities as net assets released from restrictions.

Rett Syndrome Research Trust, Inc.
Notes to Financial Statements
December 31, 2015
(See Report of Independent Auditor)

Use of Estimates

The preparation of financial statements in conformity with generally accepted accounting principles requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosures of contingent assets and liabilities at the date of the financial statements and the reported amounts of revenues and expenses during the reported period. These assumptions can include depreciable lives of long-lived assets, allocation of functional expenses and discount factors used to record long term grants payable to name a few. Actual results could differ from those estimates.

Cash Equivalents

For purposes of the statement of cash flows, the Organization considers all highly liquid debt instruments purchased with maturity of three months or less to be cash equivalents. The Organization maintains its cash accounts at various financial institutions across the State of Connecticut. Accounts are guaranteed by the FDIC up to \$250,000 per financial institution. As of December 31, 2015, the Organization is insured up to \$500,000 of recorded bank balances. The balance in excess of FDIC limitations is \$5,169,965 at December 31, 2015. The Organization has not experienced any losses in such accounts and believes it is not exposed to any significant risk in maintaining accounts with the financial institutions.

Promise to Give and Allowance for Uncollectable Accounts

Promises to give are stated net of an allowance for doubtful accounts and valuation discount. The Organization estimates the allowance based on its historical experience of the relationship between actual bad debts and receivables. As of December 31, 2015, management believes that all promises are fully collectable.

Property and Equipment

Property and equipment is recorded at cost and is being depreciated over three years.

Donated Facilities and Services

The Organization records the value of donated facilities as revenue with an equivalent amount recorded as expense. The value of this donation is \$3,000 for the year ended December 31, 2015. Contributed services that create or enhance non-financial assets and require a specialized skill are reported as contributions at their fair value with a corresponding amount recorded as an expense.

Functional Expenses

The Organization allocates its expenses on a functional basis among its program and support services. Expenses that can be specifically identified with a program or support service are allocated directly according to their natural classifications. Other expenses that are common to several functions are allocated based upon estimates made by management.

NOTE 3 – Income Taxes

The Organization has been granted tax-exempt status by the Internal Revenue Service under Internal Revenue Code Section 501(c)(3). Accordingly, no provision for federal or state income taxes has been recorded in the accompanying financial statements. Management of the Organization does not believe it has any uncertain tax positions. The Organization's tax returns remain open to examination by regulatory authorities from December 31, 2012.

Rett Syndrome Research Trust, Inc.
Notes to Financial Statements
December 31, 2015
(See Report of Independent Auditor)

NOTE 4 – Promises to Give

At December 31, 2015 and 2014, contributors to the Organization have unconditionally promised to give \$725,738 and \$1,234,629, respectively. Long-term promises have been discounted at 2% per annum which the Organization believes to be its risk free rate of return. The promised contributions are due as follows:

| | <u>2015</u> | <u>2014</u> |
|---------------------------------------|-------------------|---------------------|
| Within one year | \$ 725,738 | \$ 716,229 |
| One to five years | - | 540,000 |
| More than five years | - | - |
| Subtotal | <u>725,738</u> | <u>1,256,229</u> |
| Less: present value component | - | (10,800) |
| Less: allowance for doubtful accounts | - | - |
| Promises to give, net | <u>\$ 725,738</u> | <u>\$ 1,245,429</u> |

Management has evaluated all promises to give and believes that a reserve for uncollectable accounts is not warranted as there has never been any history of write-offs.

NOTE 5 – Grants Payable

The Organization has committed to unconditional promises to give of \$12,232,215 and \$9,513,123 as of December 31, 2015 and 2014, respectively to various research organizations over the next several years. Long-term commitments have been discounted at 2% per annum which the Organization believes to be its risk free rate of return. The timing of future grants payable is as follows:

| | <u>2015</u> | <u>2014</u> |
|-------------------------------|----------------------|---------------------|
| Within one year | \$ 6,639,332 | \$ 5,537,722 |
| One to five years | 5,623,383 | 3,975,401 |
| More than five years | - | - |
| Subtotal | <u>12,262,715</u> | <u>9,513,123</u> |
| Less: present value component | (158,279) | (86,547) |
| Grants payable, net | <u>\$ 12,104,436</u> | <u>\$ 9,426,576</u> |

Future funding of committed grants is predicated upon anticipated fundraising activities.

NOTE 6 – Concentrations

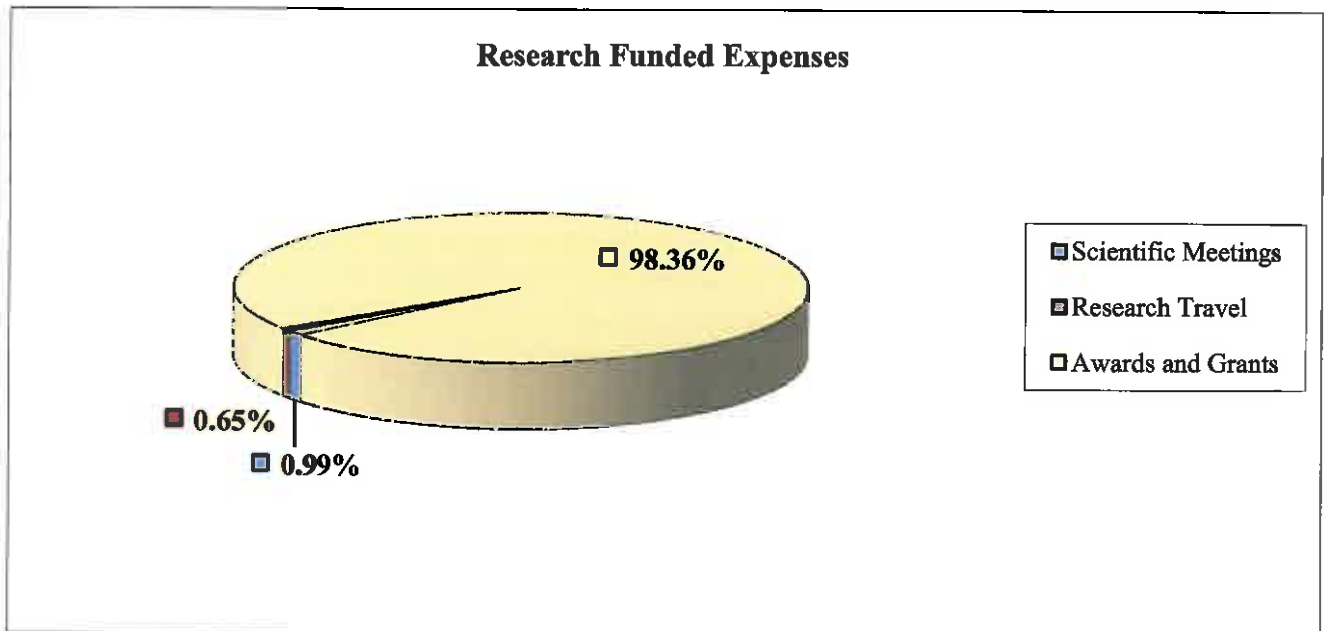
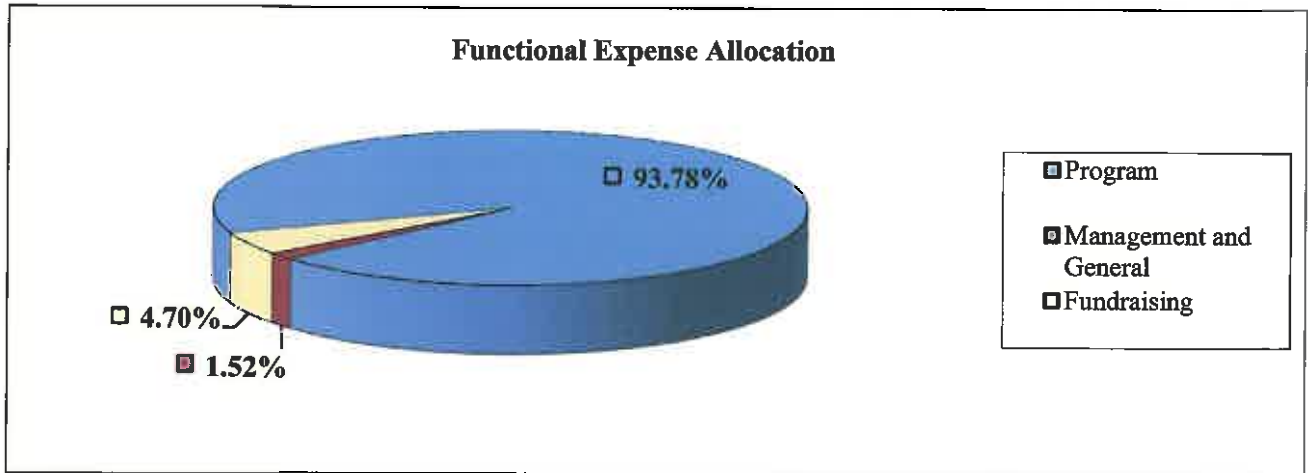
The Organization has several donors that have provided at least 10% of aggregate contributions for the year ended December 31, 2015.

Rett Syndrome Research Trust, Inc.
Notes to Financial Statements
December 31, 2015
(See Report of Independent Auditor)

NOTE 7 – Subsequent Events

In accordance with FASB Accounting Standards Codification 855, Subsequent Events, the Association has evaluated subsequent events to the Statement of Financial Position date of December 31, 2015 through April 18, 2016, which is the date the financial statements were available to be issued. Management has determined that there are no subsequent events that require disclosure.

Rett Syndrome Research Trust, Inc.
Supplemental Statement - Functional Expense Allocation and
Research Funded Expense Graphs
For the Year Ended December 31, 2015



See report of independent auditor.

Rett Syndrome Research Trust, Inc.
Supplemental Statement - Research Awards and Grant Commitments and Payments Schedule
For the Year Ended December 31, 2015

| | <u>Commitment at December 31, 2014</u> | <u>Additional Commitments</u> | <u>Payments Against Commitments</u> | <u>Commitment at December 31, 2015</u> |
|---|--|-----------------------------------|---|--|
| Hospital for Sick Kids Monica Justice | \$ 715,680 | \$ - | \$ (354,296) | \$ 361,384 |
| Fred Hutchinson Cancer Research Antonio Bedalov | 217,500 | 824,575 | (217,500) | 824,575 |
| University of Virginia Jonathan Kipnis | 165,000 | - | (165,000) | - |
| UNC - Chapel Hill Benjamin Philpot | 468,699 | - | (468,699) | - |
| Harvard University/MGH Jeannie Lee | 490,000 | - | (180,000) | 310,000 |
| UMASS Medical School Michael Green | 312,500 | - | (250,000) | 62,500 |
| Children's Hospital at Montefiore Aleksandra Djukic | 403,000 | - | (268,668) | 134,332 |
| MECP2 Consortium Harvard University of Edinburgh OHSU | 2,288,230 | - | (1,139,089) | 1,149,141 |
| Gene Therapy Consortium UNC - Chapel Hill Nationwide Children's Hospital University of Glasgow OHSU | 1,143,151 | - | (578,100) | 565,051 |
| Baylor College of Medicine - Huda Zoghbi - duplication ASO Screen | 201,250 643,699 | - | (115,000) (229,634) | 86,250 414,065 |
| Children's Hospital at Montefiore | - | - | - | - |
| UCSD Jeff Neul | 154,650 | - | (154,650) | - |
| LSRF Sung Yon Kim | 91,500 | - | - | 91,500 |
| California Institute of Technology Ali Khoshnan | 150,000 | - | (100,000) | 50,000 |
| Trinity College Daniela Tropea | 13,000 | - | (13,000) | - |
| Washington University Daniel Ory | 3,000 | - | (3,000) | - |

See report of independent auditor.

Rettsyndrome Research Trust, Inc.
Supplemental Statement - Research Awards and Grant Commitments and Payments Schedule
For the Year Ended December 31, 2015

| | <u>Commitment at December 31, 2014</u> | <u>Additional Commitments</u> | <u>Payments Against Commitments</u> | <u>Commitment at December 31, 2015</u> |
|--|--|-----------------------------------|---|--|
| Lurie Children's Hospital Debra Weese Mayer/Michael Carroll | 137,638 | - | (78,648) | 58,990 |
| UNC - Chapel Hill Terry Magnuson | 200,000 | - | (200,000) | - |
| Case Western David Katz | 271,700 | - | (193,967) | 77,733 |
| Case Western/Cleveland Clinic David Katz/Daniel Sessler | 1,295,131 | - | (158,681) | 1,136,450 |
| University of Alabama/Birmingham Lucas Pozzo Miller | 110,000 | - | (110,000) | - |
| Children's Hospital at Montefiore Aleksandra Djukic - Copaxone additional | 37,795 | - | (37,795) | - |
| CDD | - | 20,000 | (20,000) | - |
| DaimR | - | 26,815 | (26,815) | - |
| Jackson Labs | - | 42,052 | (35,208) | 6,844 |
| Children's Hospital at Montifore | - | 88,000 | (88,000) | - |
| Boston Children's Hospital Michael Fagiolini | - | 337,336 | (125,241) | 212,095 |
| University of Rochester John Foxe | - | 533,607 | - | 533,607 |
| Erasmus MC Joost Gribnau | - | 177,900 | - | 177,900 |
| Case Western David Katz (CRISPR) | - | 14,154 | (14,154) | - |
| Harvard University Jeannie Lee | - | 766,854 | - | 766,854 |
| UNC - Chapel Hill Mark Zylka | - | 400,000 | (150,000) | 250,000 |
| MIT Robotics | - | 8,000 | (8,000) | - |
| Nemours/ AI duPont Andrew Napper | - | 230,101 | - | 230,101 |
| Neorolixix | - | 530,000 | (470,000) | 60,000 |
| University of Glasgow Stuart Cobb | - | 86,208 | - | 86,208 |

See report of independent auditor.

Rett Syndrome Research Trust, Inc.
Supplemental Statement - Research Awards and Grant Commitments and Payments Schedule
For the Year Ended December 31, 2015

| | <u>Commitment at December 31, 2014</u> | <u>Additional Commitments</u> | <u>Payments Against Commitments</u> | <u>Commitment at December 31, 2015</u> |
|-----------------------------------|--|-----------------------------------|---|--|
| UTSW | | | | |
| Stephen Turley | - | 156,180 | (39,045) | 117,135 |
| OMBD Consortium | | | | |
| University of Alabama/Birmingham | | | | |
| Emory University | | | | |
| Children's Hospita; at Montefiore | | | | |
| University of Colorado | - | 4,500,000 | - | 4,500,000 |
| | <u>\$ 9,513,123</u> | <u>\$ 8,741,782</u> | <u>\$ (5,992,190)</u> | <u>\$ 12,262,715</u> |

See report of independent auditor.