



## SETBP1 SOCIETY RESEARCH GRANT APPLICATION 2018 REQUIREMENTS AND GUIDELINES

The SETBP1 Society's Research Grant Program has a two-step application process. A letter of intent is required in advance of submitting a full grant application. Only investigators who have submitted a letter of intent and have been invited to submit a formal grant application will be considered for SETBP1 Society funding. For more information on how to submit a letter of intent, please refer to the Letter of Intent Instructions document available on the SETBP1 Society website. The 2018 deadline for letters of intent is Sunday, July 22 by 5:00 p.m. central standard time.

### Background

SETBP1 disorder is a monogenetic, orphan neurodevelopmental disorder characterized by expressive language delay, autistic traits, fine/gross motor challenges, attention deficits, and cognitive disability. DNA changes that lead to a decreased amount of the SETBP1 protein are associated with SETBP1 disorder. The DNA changes are expected to be de novo. There are no approved therapies and standard of care is not effective in improving cognitive, language or motor deficits.

The following instructions are for applicants who have already submitted a letter of intent and have been invited to submit a full application to SETBP1 Society. Time extensions of grants need to be justified in writing, submitted to SETBP1 Society before the end of the grant period. Extensions are subject to approval and review by SETBP1 Society.

### Schedule for Applications

- Letter of Intent (LOI) Deadline: Sunday, July 22, 2018
- Notification of approved LOI applicants to submit full grant application: September 1, 2018
- Full Grant Application Deadline: Friday, October 5, 2018
- Grant Approval Announced By: November 30, 2018
- Funds available: December 1, 2018

### Priority areas for this grant include:

- Gene expression profiling for individuals with SETBP1 disorder
- Development of clinical, biochemical, or neuroimaging biomarkers to accelerate drug development and early diagnosis
- High throughput screening in novel cellular assays relevant to SETBP1 disorder
- Derivation and propagation of new cell lines of SETBP1 disorder that will have important research and clinical application for understanding, diagnosing and treating SETBP1 disorder.

### Application Requirements

The research grant application should be from 6-13 pages in length including the application form, the budget form, and any figures or tables. The biographical information and publication list will not be counted towards the 13-page limit. Please read the priority areas listed above before writing the project abstract, lay person summary and research plan. The grant should be in the following format: single-sided, single-spaced, 12-point font, and 1-inch margins all around with page numbers at the bottom of the sheet. The application should contain the following sections:

1. Application Form (2 pages)

The provided cover sheet should be completed and signed.

2. Project Abstract & Lay Person Summary (1-2 pages)

Briefly and clearly state the nature, objective, procedures and significance of the proposed research and explain how the study furthers knowledge on SETBP1 disorder and/or improves the treatment or diagnosis of the disease in children. The lay version of the abstract should be written for a non-medical person to understand. The lay summary may be used by SETBP1 Society for communication and publicity purposes.

3. Research Plan (2-7 pages)

The narrative should cover the following areas in detail:

- Title of project
- Background & Significance: This should provide information on the scope of the problem, summary of population or elements to be studied, a critical evaluation of previous research and existing knowledge, and insight into gaps in knowledge that the study is intended to fill. The section should clearly indicate how study findings might impact the evaluation or medical management of children with SETBP1 disorder or the impact to overall understanding of SETBP1 disorder.
- Specific Aims: This should present the broader research objectives as well as each specific study aim, rationale, hypothesis, and milestone to be tested in the project.
- Research Design & Methods: This should describe the experimental design and methods that are used to accomplish the specific aims. This section should describe how data will be collected, analyzed and interpreted. Any new methodology and its advantages over existing techniques should be mentioned. Potential difficulties and limitations of the proposed procedures and statistical methodologies along with alternative approaches should be mentioned. If the study involves human subjects, details on the study and control group criteria as well as the recruitment process should be provided.
- Timetable: This should specify the time required for study setup or patient recruitment, conducting the experiment, data analysis and evaluation, and dissemination of study findings.
- Resources & Environment: This should describe the research facility, laboratory space and major equipment needed for the study. Any procedures, materials or situation that may be hazardous should be described along with the proposed precautions to be taken.

4. Budget (1-2 pages)

- Budget breakdown: Include a itemized research budget for the proposed project stating the amount requested from SETBP1 Society along with other confirmed funding sources. Please use the specific budget form provided. If you need extra space, you can add the extra details on a separate page.

If the project costs of the study exceeds what is being requested from SETBP1 Society, please indicate other sources of funding or provide an explanation of how additional funding will be secured to complete the study.

Administrative overhead or indirect costs will not be covered by the grant and therefore should not be included in the budget. SETBP1 Society does not cover institutional construction or renovation, purchase of capital equipment other than what is needed for the proposed

research, office equipment or furniture, equipment service contracts, travel to medical meetings, tuition fees, journal subscriptions, dues or memberships, and publication fees.

- Budget justification: The narrative should explain the budget items and reason for the expense. For project personnel, an explanation of their project role should be included.

5. Research Approval Letter (1 page)

Biomedical research involving human subjects or animals require an Institutional Review Board (IRB) or Institutional Animal Care and Use Committee (IACUC) letter or equivalent letter for a non-US based institution of approval from the investigator's affiliated institution. If one is not available at the time of the application, a general institutional letter of support can be submitted. However, grant funding will be contingent on securing an IRB or IACUC letter or equivalent letter of approval prior to the start of the study.

6. Biographical Information and Publication List (appendix)

Biographical sketches for key investigators involved with the study should include name, position, education, and training in chronological order as well as research and professional experience in chronological order. The NIH biosketch format is preferred, and an example can be downloaded from <https://grants.nih.gov/grants/forms/biosketch.htm>. Only publications from the past 5 years should be listed. Letters of support are welcome, but are not required.

### **Application Submission**

A PDF copy of the proposal should be electronically submitted to [research@setbp1.org](mailto:research@setbp1.org) no later than 5:00 p.m. central standard time on Friday, October 5, 2018. The required format for submission is as follows:

- All documents should be merged into one pdf file. This includes the cover sheet, application, biosketch and supporting documents.
- Documents requiring signatures (cover sheet, IRB or IACUC letter or equivalent letter) need to be signed first, scanned and then merged into the full application file. Digital signatures will also be accepted.
- The PDF file should be saved as "investigator first and last name\_institution\_2018." An example would be to name the file "John Smith\_Univ of Texas\_2018"

No extensions of the deadline can be made. Once submitted, a confirmation email will be sent by SETBP1 Society.

In addition, one printed copy of the proposal, clipped together and not stapled, should be received no later than Wednesday, October 17, 2018. The proposal should be mailed to: SETBP1 Society, P.O. Box 301584, Austin, TX 78703 USA

For questions, please contact Haley Oyler, SETBP1 Society President, at 512-522-8072 or [research@setbp1.org](mailto:research@setbp1.org).

### **Application Review Process**

Applications are reviewed in detail by SETBP1 Society's medical and scientific advisors and are discussed as a group to determine funding priority based on SETBP1 Society's research criteria. A prioritized list is then forwarded to SETBP1 Society's Board of Directors for final funding decisions.

Each research grant is carefully evaluated on the basis of:

1. Significance & Scientific Merit

- What significant contribution will the study make to the understanding and/or treatment of SETBP1 disorder in children?
  - Is the research in-line with the research priorities of SETBP1 Society?
2. Approach & Feasibility
    - Are the conceptual framework, design, methods and analyses adequately developed, well integrated and appropriate to the aims of the project?
    - Are potential risks and problems addressed and alternative methods recommended?
    - Will the study be completed in the proposed time frame and within the proposed budget?
  3. Innovation
    - Are the aims original and innovative?
    - Does the project employ novel concepts, approaches and methods?
  4. Investigator Qualification & Environment
    - Does the investigator have the expertise and commitment to carry out the proposed work?
    - Are all the required elements such as laboratory, technology, equipment, reagents, and human or animal subjects available for the project?
  5. Ethical & Regulatory Adherence
    - Is the study ethical and does it abide by HIPAA regulations or equivalent regulations for outside the US?
    - Will the study be conducted properly in accordance to IRB and IACUC regulations or equivalent regulations for outside the US?
    - Are all conflicts of interest, including but not limited to other organizations, pharmaceutical companies, and biotech companies declared?
  6. Prospect of long term funding
    - What is the likelihood of getting subsequent NIH or other major funding to sustain the project and/or expand the research?

Final decisions on funding will be made by SETBP1 Society's Board of Directors by November 30, 2018, and grant applicants will be notified by email or U.S. mail. All decisions about grant applications made by SETBP1 Society are final and are not subject to appeal.

### **Research Grant Terms**

Before SETBP1 Society disburses funds to a grant recipient, the principal investigator (PI) will be asked to sign a funding agreement letter with the SETBP1 Society which outlines the below research grant terms:

1. Grant Period: Grants are for one year of funding beginning from the time funds are received until the same time the following year. Extensions will be considered for delays if a valid reason is submitted in writing before the end of the grant period and the SETBP1 Society Board approves.
2. Grant Payment: Upon receipt of a signed SETBP1 Society letter of agreement and letter of approval by the PI's Institutional Review Board (IRB) or Institutional Animal Care and Use Committee (IACUC) or equivalent for non-US based institutions, SETBP1 Society will award 50% of the grant award amount as start up funds. The remaining payments will be based upon meeting certain milestones and deliverables as outlined in SETBP1 Society's letter of agreement. An additional 25% of the grant award amount will be provided upon submission of the mid-year progress report. However, for studies that involve patient recruitment or acquisition of tissue/blood samples, additional payments will be contingent on the number of patients enrolled or samples acquired during the study period. Final payment is dependent on achieving the outlined aim(s) in the proposed research study, providing the

raw data findings to SETBP1 Society, submission of a detailed final report and confirmation that the research findings will be/are presented at a recognized medical conference and/or that a manuscript is expected to be accepted for publication in a peer reviewed medical or scientific journal within the year.

3. **Grant Reporting:** The awarded PI shall provide a six-month progress report which includes a 200-300 word update on accomplishments, concerns, and significant findings. The awarded PI shall provide a twelve-month progress report upon completion of the grant. This report should include an accounting summary of grant expenditures and a narrative account of what was accomplished by the grant, including a detailed description of progress made towards achieving the research goals and any significant findings. One year following the completion of the grant, the PI will be asked to provide a list of presentations, publications and/or subsequent funding received as a result of SETBP1 Society's grant support.
4. **Site of Project:** SETBP1 Society awards funding to public or private universities, hospitals and biomedical research institutions worldwide. The research shall take place only in the research facility specified in the submitted research proposal. In the event the PI leaves his or her affiliated institution, the PI shall immediately notify SETBP1 Society of the changes and submit a status report and accounting of the funds spent to date. It is the responsibility of the PI to make necessary arrangements with their institution and to obtain approval from SETBP1 Society to permit transfer of any unused SETBP1 Society research funds to the PI's new institution.
5. **Expenditures:** The grant is made only for the purposes stated in the research proposal and according to the proposed research budget. Any changes in a budget category equal to or greater than 5% of the total research grant amount must receive prior approval by SETBP1 Society before funds are expended. The grant covers direct costs only, and funds may not be used for administrative overhead, construction or renovation, purchase of capital equipment other than what is needed for the proposed research, office equipment or furniture, equipment service contracts, travel, tuition fees, journal subscriptions, dues or membership, and publication fees. At the end of the grant period, any unused funds shall be promptly returned to SETBP1 Society.
6. **Grants Administration:** The grant funds shall be physically segregated, and such funds shall be shown separately on the PI's financial records for ease of reference and verification. Records of receipts and expenditures under the grant, as well as copies of reports submitted to SETBP1 Society, shall be kept for a reasonable period of time following completion of such receipts and expenditures.
7. **Institutional Research Approval:** All SETBP1 Society-funded research projects must conform to federal regulation requiring that all institutions maintain appropriate policies and procedures for the protection of the rights and welfare of human subjects and the safe and humane treatment of animals used for research. A letter of approval from the PI's Institutional Review Board (IRB) or Institutional Animal Care and Use Committee (IACUC) must be provided to SETBP1 Society before the start of research study.
8. **Liability:** The PI assumes sole responsibility for conducting the research and supervising the work of co-investigators associated with the study and is therefore responsible for any negligent acts or omissions in connection with their research study. The PI and PI's institution shall indemnify and hold harmless SETBP1 Society, its Board, advisors, agents and constituents from any claim, judgment, award, damage, settlement, liability, negligence or malpractice arising from accepting SETBP1 Society's grant, expending or applying grant funds, or in carrying out the research study supported by the grant.
9. **Grant Suspension or Cancellation:** SETBP1 Society reserves the right to suspend or cancel the funding agreement at any time at its sole discretion for failure of the PI to abide by SETBP1 Society's grant terms. Upon receipt of a written notice of project suspension or cancellation from SETBP1 Society, the

PI should prepare and submit a project status report and accounting summary of funds expended to date. All unused funds must be returned to SETBP1 Society immediately upon its request. A PI may terminate a grant by sending written notice to SETBP1 Society stating the reason and providing a project status report and accounting summary of funds expended. The PI should make arrangements to promptly return unused funds to SETBP1 Society.

10. Dissemination of Findings: All findings resulting from research supported by SETBP1 Society shall be made public, preferably through recognized national medical meetings and peer-reviewed journal articles. Any scientific data resulting from research funded by SETBP1 Society shall be made available to other researchers and non-commercial laboratories in a timely manner at a no charge or at cost basis. All raw data and summary data resulting from research supported by SETBP1 Society shall be provided to SETBP1 Society per a signed data sharing agreement.
11. Presentation and Publication Acknowledgement: All presentations and publications related to research funded in part or in whole by SETBP1 Society's research grant must cite "SETBP1 Society" (full name of foundation) as a source of funding. SETBP1 Society shall be notified when a presentation or publication related to the funded research has been confirmed, and a PDF file of the final abstract or publication shall be forwarded to SETBP1 Society when available.
12. References to Work: Both SETBP1 Society and the PI may include general, non-confidential information about the research grant in announcements to the general public and their constituents. For specific print or internet references, such as communication materials or press releases, the PI or PI's institution must acknowledge SETBP1 Society's involvement and notify the Foundation before distribution.
13. Intellectual Property (IP): "Invention" means all discoveries, improvements, inventions, new concepts and ideas arising from the study together with all related data, results and information. Title to any Invention shall reside with the grantee institution. The grantee institution will notify SETBP1 Society promptly after identifying any invention that the grantee institution believes is patentable, and will supply SETBP1 Society with copies of that invention. For a patented invention conceived and reduced to practice that is subsequently licensed under this grant, SETBP1 Society and the grantee institution will negotiate in good faith to enter into a licensing agreement consistent with the grantee institution's policies and procedures solely for the equitable distribution of net income from the invention, taking into consideration the level of funding provided. Grantee institution shall make commercially reasonable efforts to license the invention for commercial development to a third-party. Unless otherwise agreed to by SETBP1 Society, the institution or other title holder shall agree that, if it or its designee or licensee has not taken effective steps to bring the invention to practical or commercial application through licensing or otherwise on terms that are reasonable in the circumstances within three years after issuance of the patent, SETBP1 Society shall receive an automatic grant of a non-exclusive, indefinite, fully paid-up, royalty-free, transferable, irrevocable worldwide license (with the right to sub-licenses) to advance such invention within the field of SETBP1 disorder research, diagnosis, and treatment.



## 2018 RESEARCH GRANT APPLICATION FORM

### RESEARCH INFORMATION

---

Title of Research Project: \_\_\_\_\_  
Type of Research:       Basic                       Clinical                       Translational  
Research Involvement:       Human Subjects       Animal Subjects       Biohazards

### APPLICANT INFORMATION

---

Principal Investigator Name (First, MI, Last) \_\_\_\_\_  
Title \_\_\_\_\_ Degree(s) \_\_\_\_\_  
Institution \_\_\_\_\_ Division/Department \_\_\_\_\_  
Mailing Address \_\_\_\_\_  
Phone \_\_\_\_\_ Fax \_\_\_\_\_ E-mail \_\_\_\_\_

### INSTITUTION & SUPERVISOR INFORMATION

---

If research is to be conducted at a different site:

Institution \_\_\_\_\_ Division/Department \_\_\_\_\_  
Mailing Address \_\_\_\_\_  
Phone \_\_\_\_\_ Fax \_\_\_\_\_ E-mail \_\_\_\_\_

If a supervisor or co-investigator is involved:

Supervisor Name \_\_\_\_\_ Title/Degree(s) \_\_\_\_\_  
Supervisor Phone \_\_\_\_\_ E-mail \_\_\_\_\_  
Co-investigator Name \_\_\_\_\_ Title \_\_\_\_\_  
Institution \_\_\_\_\_ Division/Department \_\_\_\_\_  
Co-investigator Phone \_\_\_\_\_ E-mail \_\_\_\_\_

### RESEARCH PROJECT BUDGET

---

Total research project budget: \_\_\_\_\_  
SETBP1 Society funding requested (\$25,000 maximum) \_\_\_\_\_  
Grant payment checks should be payable to: \_\_\_\_\_  
Grant payment checks should be mailed to (i.e. grants administration office): \_\_\_\_\_  
\_\_\_\_\_



## 2018 RESEARCH GRANT APPLICATION FORM (conti.)

### APPLICANT'S STATEMENT

---

I certify that the information contained in this grant application is accurate and not misleading. I agree to accept responsibility for the scientific direction, conduct, and financial and legal oversight of this project. I certify that I have read SETBP1 Society's grant terms and understand the grant requirements if awarded funding by SETBP1 Society.

Applicant's Signature \_\_\_\_\_

Date \_\_\_\_\_

- Application form
- Project abstract
- Lay person summary
- Research Plan
- Budget
- Biographical sketches
- Human subject IRB or Animal IACUC approvals or equivalent approvals
- Letters of support (optional)





**2018 RESEARCH GRANT APPLICATION BUDGET FORM**

**PROJECT BUDGET**

List Direct Costs Only

Project Title \_\_\_\_\_

Investigator \_\_\_\_\_ Dollar Amt Requested \_\_\_\_\_

Project Budget Start Date \_\_\_\_\_ End Date \_\_\_\_\_ Type of Grant \_\_\_\_\_

Other Funding Sources \_\_\_\_\_

**BUDGET DETAILS**

Personnel (Applicant Organization Only)

Name	Title/Position	% Effort	Salary	Fringe Benefits	Totals
	Principal Investigator				
Subtotals					
Amount Requested for Personnel					

Supplies, Equipment & Other (please itemize)

--	--

Total Request	
---------------	--

Budget Justification for Personnel, Supplies, and Other fees \_\_\_\_\_

\_\_\_\_\_

\_\_\_\_\_

\_\_\_\_\_