



**For the promotion  
and recognition  
of excellence  
in orphan disease  
treatment research**

#### The Review Committee

**Chairman:**

**Robert J. Desnick** Ph.D, M.D.  
Dean for Genetic and Genomic  
Medicine, Professor and Chair  
Emeritus Department of Genetic  
& Genomic Sciences, Mount Sinai  
School of Medicine, New York, USA

**Francesco Emma** M.D.  
Director of the Division  
of Pediatric Nephrology  
Chief of the Department  
of Pediatric Subspecialties,  
IRCCS Children's Hospital  
Bambino Gesù, Rome, Italy

**Norio Sakai** M.D., Ph.D.  
Child Healthcare and  
Genetic Science Laboratory,  
Division of Health Science,  
Osaka University Graduate  
School of Medicine,  
Osaka, Japan

**To submit an Application:**  
[prize.recordati.it](http://prize.recordati.it)

**Contacts:**  
[recordatiprize2019@recordati.com](mailto:recordatiprize2019@recordati.com)

**Recordati S.p.A**  
Via M. Civitali, 1  
20148 Milan, Italy  
Ph +39 02 48787.1  
Fax +39 02 40 073 747  
[www.recordati.com](http://www.recordati.com)

Milan, 10<sup>th</sup> September

## **The International Prize for Scientific Research Arrigo Recordati 2019**

### **CALL FOR NOMINATIONS**

The International Prize for Scientific Research was established in 2000 in memory of the Italian pharmaceutical entrepreneur Arrigo Recordati to carry on his legacy and to inspire scientists and researchers to make important discoveries benefiting people worldwide in the field of cardiovascular disease. Arrigo Recordati, who passed away prematurely in 1999, strongly believed in the power of research to drive the development of the pharmaceutical industry and provide products beneficial to public health and individual wellbeing.

In the last ten years Recordati made the Rare Diseases field a health priority and increased its commitment worldwide through its dedicated subsidiaries, Orphan Europe and Recordati Rare Diseases, to research, develop and market a number of treatments for orphan diseases.

To reflect its commitment, Recordati decided that the 2019 Edition of The International Prize for Scientific Research Arrigo Recordati will be dedicated to **the promotion and recognition of excellence in orphan disease treatment research.**

**International projects within the area of rare/orphan disease treatments in all therapeutic areas (except for oncology, hematology and immunology) are eligible to apply as long the disease concerned has a prevalence of not more than 1 in 2000.**

The 2019 Award is open to researchers of all nationalities who are not in any way directly affiliated with pharmaceutical or medical device companies.

The 2019 Award will be given in recognition of ongoing research projects, for which preliminary results, such as proof-of-concept and proof-of-principle, have already been obtained.

**The winner project will receive a research grant of € 100,000.**

Applications will be reviewed by an independent panel of internationally recognized experts who have provided leadership throughout their long careers in the field of Rare Diseases.

## 2019 Review Committee

For the ninth edition of the Award, the Review Committee is composed of:

**Robert J. Desnick Ph.D, M.D.** **Francesco Emma M.D.**

Dean for Genetic and Genomic Medicine, Professor and Chair Emeritus Department of Genetic & Genomic Sciences, Mount Sinai School of Medicine, New York, USA

Director of the Division of Pediatric Nephrology Chief of the Department of Pediatric Subspecialties, IRCCS Children's Hospital Bambino Gesù, Rome, Italy

**Norio Sakai M.D., Ph.D.**

Child Healthcare and Genetic Science Laboratory, Division of Health Science, Osaka University Graduate School of Medicine, Osaka, Japan

Although Recordati recognizes the value of fundamental research aimed at unraveling the disease pathophysiology/etiology, **projects on orphan diseases which do not make use** (or assess the potential) **of an experimental treatment for a rare disease are not eligible for the Award.**

If the experimental treatment is based on a pre-existing treatment, the project is only eligible if the treatment considered is being repurposed in another indication or if its formulation/administration changed in a major way, in order to improve treatment quality, compliance, efficacy, safety or any other additional patient's benefits.

Projects concerning both **medicinal products and medical devices** are eligible for the Award as long as the disease treated is rare.

Intellectual Property Rights, when present, will be safe-guarded.

## 2019 Award Ceremony

The winner project will be announced during an Award Ceremony at the Society for the Study of Inborn Errors of Metabolism Congress – (2019 SSIEM, Rotterdam, 2<sup>nd</sup>-6<sup>th</sup> September 2019).

The winner project will receive a research grant of € 100,000.

There will be no University or Institutional indirect costs as a condition for this Award.

## 2019 Nominations Procedure

Nominations procedure will follow a two-step selection approach:

**1) Preselection: from 10<sup>th</sup> September 2018 to 15<sup>th</sup> December 2018**

- Applications will be processed solely on-line and must be submitted in English.
- All applications submitted in the preselection period and which will satisfy the eligibility criteria (*see p. 3*) will be considered.
- Applicants should submit an initial proposal composed of a Summary Page (*see p. 4*) and a Letter of Intent (*see p. 5*) describing the proposed project in 1200 words (plus 1 page for figures and/or tables) by uploading the requested documentation at: **prize.recordati.it**
- Applicants who submit an initial proposal will receive an email confirming receipt.

Members of the Review Committee will evaluate all the initial proposals to determine which Applicants will be invited to submit Full Applications.

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## 2) Full Applications: from 15<sup>th</sup> March 2019 to 30<sup>st</sup> April 2019

- Selected Applicants will be invited to submit a Full Application uploading the documentation at: **prize.recordati.it**

All members of the Review Committee will assess and select the winning project according to the pre-specified Award criteria, quality of the research as well as the therapeutic impact of the results.

The Award has “code of conduct” guidance that ensure reviews are conducted in a fair and equitable manner. Applications in which reviewers, or institutions with which they are affiliated, have an interest, will not be considered.

The members of the Review Committee are bound by the confidentiality rules generally applicable to such bodies.

The Review Committee’s decision is final and there is no appeal mechanism.

Applicants who have constructive suggestions about the review of their Applications, or the process in general, are encouraged to write to: **recordatiprize2019@recordati.com**

## 2019 Award Criteria

### Eligibility criteria:

- Applied research projects on rare disease treatment (preclinical or clinical) projects have to make use of an experimental treatment approach to be eligible
- Ongoing projects with interim results or clear milestones in order to gather sufficient data to demonstrate the effectiveness/safety
- If the therapeutic agent already exists, the project is only eligible if the treatment is being repurposed for another rare indication or if the treatment was reformulated in order to provide an added benefit for the patient (easier to use, better compliance, safety, efficacy) or if the treatment is being used in combination with other treatments or devices.
- Applicant CV needs to be included.
- Applicants have to make use of the Application Form and to comply with instructions (*see p. 4*).

### Exclusion criteria:

- Research projects aiming at developing or characterizing new models
- Fundamental research projects which do not assess or make use of any experimental treatment or device
- Studies with only results obtained *in vitro* cellular models
- Oncology, hematology or immunodeficiency projects
- Projects already awarded a Prize in the past
- Projects sponsored by other pharmaceutical or medical device companies
- Projects completed
- Projects without Applicant’s CV
- Projects non-compliant with the Application Form.

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School of Medicine,  
Osaka, Japan

## Application Form

The Application Form must include a Summary Page and a Letter of Intent.

The steps to apply are the following:

**1) Preselection of the initial proposals based on Summary Page and Letter of Intent.**

Compliance with the following Summary Page and Letter of Intent provided below are required for Award consideration.

If the Application Form received does not contain the items mentioned below, it will not be considered.

**2) The Applicants of the selected initial proposals will be invited to submit Full Applications to compete for the Award.**

The selection of the winning project will be based on the evaluation of the Review Committee according to the pre-specified Award criteria, quality of the research as well as the therapeutic impact of the results.

## Summary Page

<b>PRODUCT/ COMPOUND</b>	
<b>DEVELOPMENT PHASE</b>	
<b>PROJECT TITLE</b>	
<b>APPLICANT - INSTITUTION</b>	
<b>CONTACT DETAILS</b>	
<b>APPLICANTS(s) CURRICULUM VITAE (upload files)</b>	

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## Letter of Intent

The Letter of Intent is the project description.

In 1200 words (plus 1 page for figures and/or tables), please succinctly address the relevant information of the initially proposed project including:

- 1) background, unmet need and significance of the project;
- 2) specific aims, objectives, or hypothesis;
- 3) preliminary studies and data relevant to the proposed project;
- 4) experimental design: description of methods and analyses to be used for proof of concept study, proposed clinical trial design (primary & secondary endpoints), or design of novel device for treating a disease or monitoring therapeutic effectiveness;
- 5) expected results and expected timeframe of the results;

Please, feel free to add any additional information/supportive data, as needed.

## 2018/2019 Award Calendar

10 <sup>th</sup> September/ 15 <sup>th</sup> December 2018	Submission of initial proposals (Summary Page and Letter of Intent)
January/February 2019	Review and preselection of the initial proposals
15 <sup>th</sup> March/30 <sup>th</sup> April 2019	Submission of Full Applications
May/June 2019	Review of Full Applications
2 <sup>nd</sup> /6 <sup>th</sup> September 2019	Award Ceremony, SSIEM 2019, Rotterdam

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