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## The I-GENE project:

a new concept of genome editing



*Dear Readers,*

*The I-GENE team is pleased to welcome you to the 1<sup>st</sup> Newsletter. In this inaugural issue, we would like introducing you to the I-GENE project.*

*As you know, the 21<sup>st</sup> century is facing with extraordinary opportunities of the genome editing revolution, thanks to the discovery of new tools to precisely manipulate genomes of cells or organisms. At the same time, however, they imply an increasing rise of ethical concerns. In this context, I-GENE would develop a new concept of genome editing to expand the biotech and the therapeutic applications, improving the efficiency, specificity and the safety profile of the methodology, We invite you to join us on our journey!*

*Please, stay connected with us for more updates about our science in the upcoming issues!*

*Thank you and enjoy your read*

**Prof. Vittoria Raffa**  
**I-GENE coordinator**

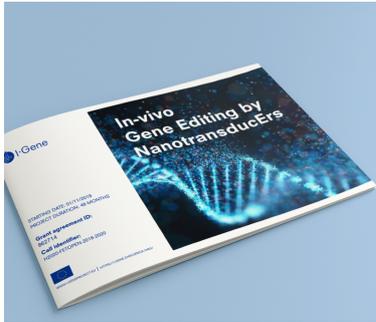
**[#SAVETHEDATE](#)**

I-GENE SUMMER SCHOOL (Pisa, IT – 28<sup>th</sup> June – 1<sup>st</sup> July 2021)

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## THE I-GENE MISSION

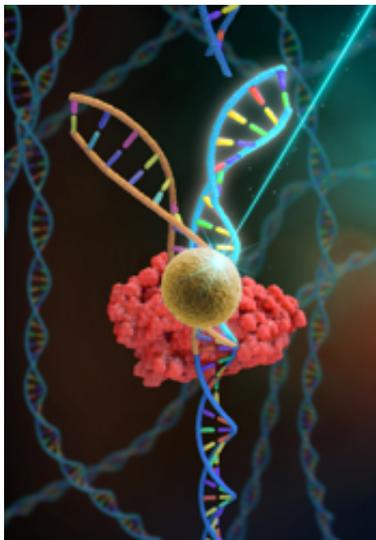
I-GENE technology would push the boundaries for efficient and reliable approaches to introduce precise, targeted changes to the genome of



living cells, the longstanding and main goal of gene therapy and biomedical research.

## THE RESEARCH TEAM

Teamworking is the source of our strength. The I-GENE team consists of 5 partners (2 from the academy sector and 3 from the industrial sector), recognized for their excellence in the field of gene therapy, nanomedicine, photonics and material science. Each team is the leader of a specific work package. Nevertheless, the single work packages display an interrelating structure, favouring strong cooperation of the different groups



## THE I-GENE CONCEPT

Although CRISPR/Cas9 and enzyme-based editors hold promise for genome surgery by erasing harmful mutations while rewriting them in helpful ones, they still face critical barriers related to delivery and safety. Our methodology relies on the development of a Cas9 nanoformulation, which triggers a light-switchable DNA double-strand break or cleavage.

### Why has the I-GENE tech superior delivery properties?

The I-GENE vector has the ability of spontaneous nuclear localization of the editing machinery.

### Why does I-GENE tech offer a superior safety profile?

Thanks to an approach of synthetic biology, I-GENE tech implements a concept of multi-input AND gates, where the output (gene editing) is true only if multiple inputs are simultaneously true, making off-target events unlikely to occur.

[See our explanatory video](#)

## WHAT IT'S BREWING IN THE I-GENE LABS?

During this first year, the PROCHIMIA partner developed a nano-formulation of Cas9 using an approach of link chemistry for precise supramolecular control of surface coverage densities and bond orientation. The conjugate was validated in human melanoma cell lines and zebrafish embryos, demonstrating high editing efficiency, spontaneous cell internalization and nuclear translocation. The IIT partner is modelling the optical properties of the photo-switchable nano-formulation, while the partners Msquared and Lionix have designed and released the first prototypes of the laser set-up and the lab-on-chip for radiation experiments. For additional details about the I-GENE research, please, meet I-GENE scientists in our next public appointment, the I-GENE summer school.

## WHAT NEXT?

The big challenge of the I-GENE project is to gain control on the spatio-temporal activation of the cleavage activity on-target, while minimizing off-target events. The fulfilment of this objective could expand the range for therapeutic applications, making the impossible possible, i.e., the unique recognition of a specific genomic target from any potential off-target, given in the 3 billion base

pairs of the human genome!  
Please, follow our progress in the next newsletter!

## FIRST YEAR: MANY ACCOMPLISHMENTS!!



THE KICK-OFF MEETING

The I-GENE project was presented to the general public in Pisa (Italy) in November 2019, in an old church serving nowadays as an exhibition space, connecting science with culture.



COVID PANDEMIA

Despite the COVID pandemic emergency, we were able to adapt quickly to the new reality and fulfil the commitments of the 1<sup>st</sup> year.



FIRST I-GENE TRAINING SCHOOL

The 1<sup>st</sup> I-GENE training school was held on-line with a rich agenda and content. The excellence of the speakers and the 190 participants enrolled from different countries and many disciplines, created an international atmosphere and a constructive dialogue across different domains such as genome editing, nanomedicine and ethical issues of gene therapy.



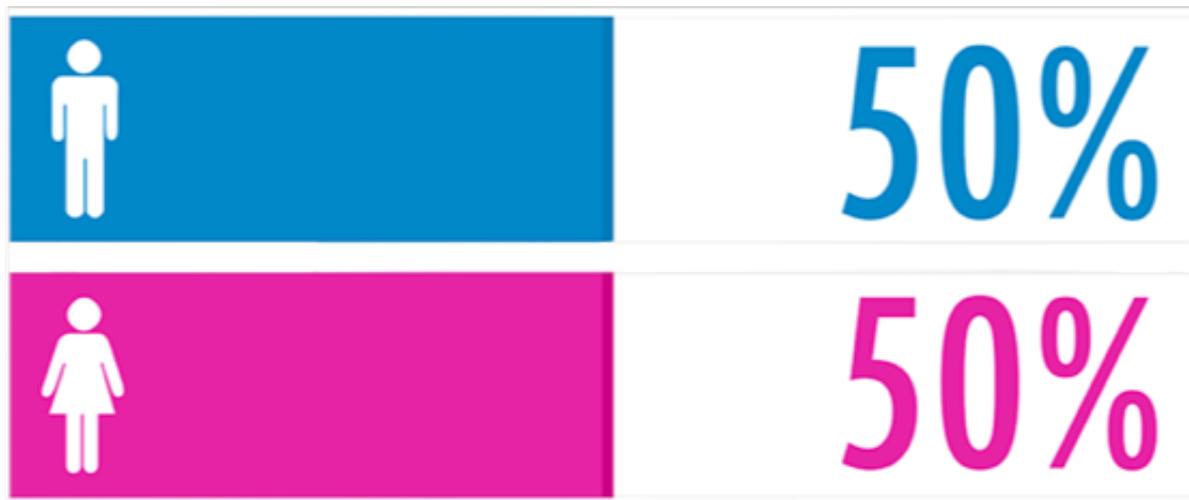
ETPN 2020

The I-GENE technology was presented in the 15<sup>th</sup> annual event of the European technology platform on Nanomedicine (ETPN2020) by Prof. V.Raffa. This virtual conference allowed introducing our project to a large audience with an academic and industrial background, to further boost our innovation and networking on a global level.

## KEY INNOVATOR

I-GENE project has been selected as KEY INNOVATOR for the European Commission, 2020

## I-GENE HAS A PERFECT GENDER BALANCE



## NANOMEDICINE FOR GENE THERAPY

### I-GENE SUMMER SCHOOL (Pisa, IT - 28<sup>th</sup> June - 1<sup>st</sup> July 2021)

Please, do not miss the ROUND TABLE on “Ethical and legal issues of gene therapy and genome editing”. Are you interested in the hot topic of nanomedicine and how it contributes to recent and future advances in the field of gene therapy? Please, meet I-GENE scientists during the I-GENE summer school that will be held in summer of 2021 in Pisa. Here, we will provide a platform for discussing the current state of knowledge, while encouraging creativity and cooperation among different audiences.

We also aim to stimulate international networking and enhance the scientific and technological exchange among academy, users and stakeholders.

Looking forward to meeting you soon.

**I-GENE Summer School**  
Pisa, 28<sup>th</sup> June / 1<sup>st</sup> July 2021  
4 Days  
**NANOMEDICINE FOR GENE THERAPY**

**DAY 1 MONDAY 28<sup>th</sup> JUNE**

9:00 - 11:00	Introduction to nanomedicine for gene therapy? <b>Prof. Marco Ballini</b> , University of Pisa (IT), Department of Biology
12:00 - 12:30	CRISPR/Cas9 technique: suspension gene editing and beyond? Part I <b>Dr. Chiara Gubellini</b> , University of Pisa (IT), Department of Biology
13:30 - 14:30	Lunch break
14:30 - 16:00	CRISPR/Cas9 technique: suspension gene editing and beyond? Part II <b>Dr. Chiara Gubellini</b> , University of Pisa (IT), Department of Biology
16:30 - 18:00	CRISPR/Cas9 technique: suspension gene editing and beyond? Part III <b>Dr. Chiara Gubellini</b> , University of Pisa (IT), Department of Biology

**DAY 2 TUESDAY 29<sup>th</sup> JUNE**

9:00 - 11:00	"The meaning of gene therapy: present and future perspectives" <b>Prof. Roberto Passini</b> , University of Pisa (IT), Department of Medicine
11:30 - 12:30	"The meaning of gene editing: CRISPR/Cas9" <b>Dr. Alessandro Giamberini</b> , University of Pisa (IT), Department of Medicine
13:30 - 14:30	Lunch break
14:30 - 16:00	"CRISPR/Cas9 in cancer and disease prevention" <b>Prof. Roberto Passini</b> , University of Pisa (IT), Department of Medicine
16:30 - 17:00	"An in vivo application of gene editing: the mouse model of retinitis" <b>Dr. Paolo Scaramia</b> , University of Pisa (IT), Department of Medicine

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**DAY 3 WEDNESDAY 30<sup>th</sup> JUNE via Nanomedicine & Nanotechnology**

9:00 - 10:00	"From nanomedicine to drug delivery systems" <b>Dr. Barbara Witz</b> , Provincia Autonoma (IT)
10:30 - 11:30	"Regulatory requirements for CRISPR/Cas9" <b>Dr. Francesco Nicolini</b> and <b>Dr. Francesco De Gregis</b> , Azienda Ospedaliera di Neurologia (IT)
11:30 - 12:30	"Introduction to photonic flow control: how to control flow light?" <b>Dr. Diego Ares</b> , Lazio (IT)
13:30 - 14:30	Lunch break
14:30 - 16:00	CRISPR/Cas9: applications in gene editing <b>Dr. Roberto Passini</b> , University of Pisa (IT), Department of Medicine

**DAY 4 THURSDAY 1<sup>st</sup> JULY**

9:00 - 10:00	"Ethical and legal issues of gene therapy and genome editing" <b>Public event</b>
10:30 - 11:30	"From nanomedicine to drug delivery systems" <b>Dr. Barbara Witz</b> , Provincia Autonoma (IT)
11:30 - 12:30	"The meaning of gene editing: CRISPR/Cas9" <b>Dr. Alessandro Giamberini</b> , University of Pisa (IT), Department of Medicine
13:30 - 14:30	Lunch break
14:30 - 16:00	Introduction to "CRISPR/Cas9" <b>Prof. Marco Ballini</b> , University of Pisa (IT), Department of Biology
16:30 - 17:30	Round Table - Open discussion - 16

Discover more

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## I-GENE Consortium



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The I-GENE project is funded by EU (grant agreement ID: 862714) under the FET-OPEN scheme of HORIZON 2020, fostering novel ideas for radically new technologies.

Please follow our social media and website to get updates on I-GENE mission and research activities:



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