

CURRICULUM VITAE

Carlo Toniatti, MD, PhD

Work Address: IRBM S.p.A. Via Pontina k. 30,600, 00071, Pomezia, (RM), Italy

Home Address: Viale dell'Astronomia 13, 00140, Roma, Italy.

Tel: +39 06 91093445 (work); +39 3420701578 (cell phone)

E-mail: c.toniatti@irbm.com (work); carlotoniatti@gmail.com (private)

QUALIFICATIONS SUMMARY

- Experience in basic research, drug discovery and clinical development in both Biotech and Pharmaceutical Company environment; solid expertise in Drug Discovery and Development (small molecules and biologics), Oncology and Gene Therapy.
- Strong familiarity with leading drug discovery programs, in both industry and academic environment, from target identification/validation to clinical development and FDA approval and with executing on translational research.
- Experienced in the management of external collaborations and alliances, in portfolio management and in managing interaction and collaborations between industry and academia.
- Have successfully led multiple programs and cross-functional teams; functions effectively in a team environment; proficient in the direct and matrixed supervision of people.
- Strong experience in leading drug discovery and development efforts in industry and in academic environment

EDUCATION

1993 **Ph. D. in Molecular and Cellular Biology and Pathology**
University of Naples, 2nd School of Medicine, Naples, Italy
Liver specific gene-transcription

1987 **M.D.: Doctor Degree in Medicine (with honors)**
University of Naples, 2nd School of Medicine, Naples, Italy

PROFESSIONAL EXPERIENCE

Nov. 1990-present

- 2018-present: IRBM S.p.A, Pomezia, Rome Italy.
- 2014-2018: Oncology Research for Biologics and Immunotherapy translation (ORBIT), MD Anderson Cancer Center, Houston, US.
- 2012 -2014: Institute for Applied Cancer Science (IACS) & Center for Co-Clinical Trial (CCCT), MD Anderson Cancer Center, Houston, US.
- 2011-2012: Institute for Applied Cancer Science (IACS), MD Anderson Cancer Center, Houston, US.
- 2009-2011: MRL Labs, Boston, US.

- 2000-2009: Merck Research Labs (MRL), Rome, Italy (I.R.B.M., fully owned by Merck from 2000)
- 1990-2000: I.R.B.M. - Istituto di Ricerche di Biologia Molecolare, Pomezia, Rome, Italy (Joint Venture between Merck/MRL and Sigma-Tau)

EMPLOYMENT HISTORY

2018-Present	Chief Scientific Officer, IRBM
2015- 2018	Executive Director, Head of Research at ORBIT (MDACC)
2013-2014	Head of Research at IACS & CCCT (MDACC)
2011-2012	Head of Research IACS (MD Anderson Cancer Center, MDACC)
2008-2011	Director (MRL)
2003-2007	Senior Investigator (MRL)
2000-2002	Senior research Fellow (MRL)
1997-1999	Research Fellow (IRBM)
1995-1997	Senior Research Biologist (IRBM)
1992-1995	Research Biologist (IRBM)
1990-1992	Staff Biologist (IRBM)

Current Position

- 2018-present** Chief Scientific Officer & VP Translational & Discovery Research at IRBM ((Integrated Research in Biotechnology and Medicine).
- Responsible for the overall scientific strategy, technological advancement, portfolio management, scientific execution, establishment and management of scientific alliances.
 - IRBM is a Partner Research Organization (total of about 160 scientists, 200 total FTEs) with decades of experience in drug discovery and development. IRBM provide support across the entire drug discovery process from target ID to clinical candidate selection for therapeutic small molecules, peptides and monoclonal antibodies.
 - In terms of Internal R&D, programs in Oncology and Infectious diseases (Viral Diseases) have been started in 2018. In December 2021, IRBM has out-licensed a novel series of fourth-generation capsid assembly modulators (CAMs) for the treatment of HBV. The CAMs were originated and developed at IRBM in alliance with Istituto Nazionale Genetica Molecolare (INGM) and San Raffaele Hospital (OSR).

Last previous positions

2015-2018

- Executive Director and Head of Research at the Oncology Research for Biologics and Biologics and Immunotherapy Translation (ORBIT) institute.
- Responsible for the overall strategy, portfolio management, scientific execution and alliances management. ORBIT is an organization (total of 30 FTEs) within the MD Anderson Cancer Center, Houston, Texas, that was established in January 2015: its mission is to guide, inform, accelerate and execute the translation of novel discoveries into clinically relevant cancer bio-therapeutics.
- The IND for the anti-OX40 agonist mAb (now GSK3174998) was filed in July 2015. Phase I in monotherapy started in October 2015. Phase I in combination with PD-1 (Keytruda, Merck) started in 2016.
- In April 2015, signed an option agreement with Astellas Pharma Inc. to research and develop a new TCR-like mAb, namely 8F4, for the treatment of patients with Acute Myeloid Leukemia (AML). The agreement also includes up to \$26 million as an option premium and for research and development funding. Phase I trial started during the 2Q2019.
- Strategic alliances with two mAb biotech companies (Kymab and Morphosys) were established
- Two small molecule programs (OXPHOS inhibitor, IACS-10759) developed at IACS under my leadership (namely (OXPHOS inhibitor, IACS-10759 and Glutaminase I inhibitor, IPN60090) entered phase I of clinical development during the 3Q2016.
- Voting Member of the MDACC Immunotherapy Platform Executive Committee.

2012-2015

- Head of Research at the Institute for Applied Cancer Science (IACS).
- Head of Research at the Center for Co-clinical trials (CCCT)
- Continued to lead IACS basic research and translational medicine efforts. Focus on metabolism and epigenetic targets.
- Established a co-development and milestone driven agreement with GSK (potential for up to \$335M revenues + royalties) for anti-OX40 monoclonal antibodies (immune checkpoint agonist). - Responsible for the scientific execution of the project at IACS/; member of the Joint Steering Committee for program development.
- Responsible for the overall scientific direction and operations of the CCCT, a pre-clinical platform whose mission is to integrate MDACC expertise and resources required to accelerate *in vivo* pre-clinical evaluation of oncology drugs in the appropriate pre-clinical models to inform the design and implementation of clinical trials. Cross functional oversight of the work of +20 pharmacologists and biologists to establish *in vivo* mouse models, design and execute pharmacokinetic, toxicity and *in vivo* efficacy studies and to generate and validate biomarker hypothesis.

2011-2012

- Head of Research at the Institute for Applied Cancer Science (IACS) at the MD Anderson Cancer center.
Lead IACS Basic Research and Translational Medicine efforts to identify and validate cancer targets and to support small molecules development programs. Lead biology teams and coordinate cross-functional biology and chemistry teams (total of > than 50 FTEs) for integrated programs to advance small molecule inhibitors into the clinic.

2003-2011

Merck – increasing responsibilities

- Lead a group of 10-50 cancer biologists engaged in 1) small molecule drug discovery efforts for an undisclosed target and 2) translational medicine efforts (responder identification, biomarkers development and selection of clinical indications) to support the clinical development of five Merck Oncology compounds..
- Cross functional oversight of the work of pharmacologists and/or biologists/biochemists and/or chemists belonging to different functional areas to ensure the successful completion of our research activities in support of oncology.
- Chair of the EDT (Early Development Team), a large (+ 30 team members, >100 indirect FTE's) company-wide multidisciplinary team responsible for the clinical development of PARP inhibitor niraparib (MK-4827). After achieving clinical proof-of-concept in a Phase 1b, Niraparib was licensed out to Tesaro in 2011. On March 2017 U.S. FDA has approved niraparib (ZEJULA) for the maintenance treatment of adult patients with recurrent epithelial ovarian, fallopian tube, or primary peritoneal cancer who are in complete or partial response to platinum-based chemotherapy.
- Biology leader and member of the EDTs responsible for the clinical development of Wee1 inhibitor (MK-1775, now AZD1775, Phase 2) and HDM2 inhibitor (MK-8242, Phase 1) and CHK1 (MK-8776, phase 1).
- Co-lead a company-wide multidisciplinary team whose goal was the identification of the best combinations of targeted agents for the therapy of biomarker-defined tumor subtypes.
- As part of the senior leadership team, help shape global portfolio, define organization structure and recruit talents to MRL sites.
- Member of the Early Stage and Late Stage Review Licensing Committees in charge for evaluating external Oncology compounds at early (pre-clinical-Phase1) or late (phase 2-3) stage of developments. Participate in due diligence efforts for in-license candidates.
- Leader of the Cell Cycle and DNA Repair Oncology area at Merck
- Member of the Merck Oncology Target Identification and Validation team.
- Initiate and maintain collaborations with academia, clinicians, biotech companies and CROs to augment internal research and explore novel preclinical and clinical strategies.

- Member of the Senior Leadership Team, a group of about 12 directors at the Boston site with governance of non-research activities ongoing at the site.

Summary of past accomplishments (Oncology):

- Led the PARP program from target identification and validation to phase 1b Proof-of concept trial with lead PARP-1/-2 inhibitor MK-4827. Also led the cross-functional team who developed an undisclosed back-up PARP inhibitor clinical candidate.
- Led a cross-functional team that developed and validated an antibody-drug conjugate platform to deliver potent cytotoxic agents linked via cleavable linkers to mAbs.
- Led a multi-disciplinary, company-wide team that explored targeted systemic and/or intratumoral delivery of siRNAs as a therapeutic approach for cancer.
- Identified and validated an undisclosed Merck Oncology target and started the corresponding small molecule drug discovery program; conceived, developed and coordinated the execution of the whole screening strategy, including hit identification and triage, research operating plan and critical assays for screening and hit validation/lead optimization.
- Provided strategic and technical leadership to different interdisciplinary program teams pursuing the development of targeted therapies for cancer.
- Introduced and validated in Merck the use of lentivirus-mediated delivery of inducible shRNA expression cassettes as a powerful tool for target validation/identification.
- Co-led high-throughput genome wide siRNA screenings to identify novel targets and/or hits sensitizing to existing Merck drugs; conceived and design follow-up strategy and assays for hits validation
- Involved in a number of strategic initiatives/teams for Merck Oncology including: development of pre-clinical oncology models, prioritization of targets for drug discovery, assessment/evaluation/licensing of novel technologies capable to help the drug discovery process.

1997-2002

GENE THERAPY

Summary of past accomplishments

- Group and Program leader (3-5 direct reports) in the context of Merck-wide project aimed at the development of novel vectors for gene delivery and regulation.
- Developed and optimized two novel transcription regulatory switches for *in vivo* ligand-dependent gene transcription regulation.
- Used transcription regulatory switches in appropriate animal models for the successful gene therapy with viral and non-viral vectors of diseases like Anemia and Proliferative Diabetic Retinopathy.
- Co-developed and optimized systems for efficient intramuscular delivery of genes by *in vivo* electro-transfer.
- Characterized the mechanisms underlying site-specific integration in human chromosome 19 of Adeno-associated virus (AAV)

- Co-developed a hybrid adenovirus (Ad)/adeno-associated virus (AAV) vector which combines the large cloning capacity of Adenovirus with the site-specific integration in chromosome 19 of AAV.

1991-1996

RECOMBINANT GROWTH FACTORS

Summary of past accomplishments

- Group leader (1-3 direct reports) within the IL-6 program at IRBM, whose goal was the generation of IL-6 variants with antagonistic activity for the therapy of IL-6 associated diseases, such as multiple myeloma and rheumatoid arthritis.
- Contributed to the characterization of the interaction mode of IL-6 with its receptors. First reported experimental evidence proposing the hexameric structure of the IL-6 receptor complex.
- Generated by site-directed mutagenesis soluble forms of human IL-6 receptor (shIL-6R) with antagonistic activity.
- Generated human interleukin-6 (IL-6) variants with > 70-fold enhanced affinity for its binding receptor by a combination of homology modeling, site-directed mutagenesis and selection of high affinity variants of IL-6 from phage-displayed libraries of mutants.
- Contributed to the generation of hIL-6 variants with super-antagonistic activity (i.e. increased affinity for the binding receptor but .no capability to trigger heterodimerization with the transducing receptor unit)
- Contributed to the generation by rational design and phage display selection of a 61 residue polypeptide (minibody) inhibitor of IL-6
- Contributed to the demonstration for the first time that IL-6 system plays a positive role in local inflammatory reactions by amplifying leukocyte recruitment.

ACADEMIC HISTORY

- **PhD student, 2nd School of Medicine, Naples, Italy, Department of Biochemistry and Biotechnology, Laboratory of Prof. Gennaro Ciliberto**
 - Identified cis- and trans- acting element required for the inducible and liver specific expression of inflammatory genes, such as the human C-reactive protein (CRP) gene.
 - Elucidated the functions of the liver specific transcription factor HNF-1 and demonstrated for the first time its transcriptional activating properties capabilities in cells.
- **1986-1987 Undergraduate student at the Department of Internal Medicine, 2nd Medical School of Medicine, Naples, Italy**
 - Internist for 18 months in the department of Internal Medicine as part of the training to obtain the Doctor Degree in Medicine
 - Plus three months in the Department of Psychiatry and three months in the Department of Obstetrics and Gynecology
- **1984-1986 Undergraduate student at the International Institute of Genetics and Biophysics (IIGB), Naples, Italy, Molecular Neurobiology Section, Laboratory of Prof. A. Giuditta.**
 - Studied macromolecules synthesis (nucleic acids and proteins) in brain during memory and learning processes.
- **Undergraduate student at the Department of Immunology, Laboratory of Prof. S. Zappacosta. 2nd School of Medicine, Naples, Italy**
 - Studied the association between autoimmune diseases and HLA haplotypes.

ACADEMIC EXPERIENCE

From 1997 to 2008 Teaching course (20 hours) of Molecular Biology & Gene Therapy for undergraduate students at the University “La Sapienza” of Rome as part of the official course for MSc in Biology

PROFESSIONAL AND ACADEMIC HONORS

- 1995 Young Investigator Award (First Place) from the International Cytokine Society (September, 1995 Harrogate, England).
- Featured in the 2020 Medicine Maker’s Power list, Biopharmaceuticals category.
- Ad-hoc reviewer for several peer-reviewed journals including Cancer Discovery, Journal of the National Cancer Institute, Cancer Research, Molecular Cancer Therapeutics, PNAS (Proceedings of the National Academy of Sciences of the United States of America), Trends in Molecular Medicine, Molecular Therapy, Cell Death & Differentiation, Human Gene Therapy and Gene Therapy
- Author of invited reviews and book chapters
- H-Index: 49; citations: 9779; total IF: 113

PUBLICATIONS

1. Simona Ponzi, Federica Ferrigno, Monica Bisbocci, Cristina Alli, Jesus M Ontoria, Alessia Petrocchi, **Carlo Toniatti**, Esther Torrente. Direct-to-biology platform: From synthesis to biological evaluation of SHP2 allosteric inhibitors. *Bioorg. Med. Chem. Lett.* 100:129626, 2024.
2. Petrocchi A, Grillo A, Ferrante L, Randazzo P, Prandi A, De Matteo M, Iaccarino C, Bisbocci M, Cellucci A, Alli C, Nibbio M, Pucci V, Amaudrut J, Montalbetti C, **Toniatti C**, Di Fabio R. Discovery of a Novel Series of Potent SHP2 Allosteric Inhibitors. *ACS Med Chem Lett.*, 14(5):645-651, 2023.
3. Torrente E, Fodale V, Ciammaichella A, Ferrigno F, Ontoria JM, Ponzi S, Rossetti I, Sferrazza A, Amaudrut J, Missineo A, Esposito S, Palombo S, Nibbio M, Cerretani M, Bisbocci M, Cellucci A, di Marco A, Alli C, Pucci V, Toniatti C, Petrocchi A. Discovery of a Novel Series of Imidazopyrazine Derivatives as Potent SHP2 Allosteric Inhibitors. *ACS Med Chem Lett.*, 14(2):156-162, 2023
4. Giacomo Paonessa, Giulia Siciliano, Rita Graziani, Cristiana Lalli, Ottavia Cecchetti, Cristina Alli, Roberto La Valle, Alessia Petrocchi, Alessio Sferrazza, Monica Bisbocci, Mario Falchi, **Carlo Toniatti**, Alberto Bresciani, Pietro Alano. Novel gametocyte-specific and all-blood-stage transmission-blocking chemotypes from high throughput screening on Plasmodium falciparum gametocytes. *Commun. Biol.* 6;5(1):54, 2022
5. Colarusso S, Ferrigno F, Ponzi S, Pavone F, Conte I, Abate L, Beghetto E, Missineo A, Amaudrut J, Bresciani A, Paonessa G, Tomei L, Montalbetti C, Bianchi E, **Toniatti C**, Ontoria JM. SAR evolution towards potent C-terminal carboxamide peptide inhibitors of Zika virus NS2B-NS3 protease. *Bioorg. Med Chem.* 1, 57 116631 (2022)
6. Biferali B, Bianconi V, Perez DF, Kronawitter SP, Marullo F, Maggio R, Santini T, Polverino F, Biagioni S, Summa V, **Toniatti C**, Pasini D, Stricker S, Di Fabio R, Chiacchiera F, Peruzzi G, Mozzetta C. Prdm16-mediated H3K9 methylation controls

fibro-adipogenic progenitors identity during skeletal muscle repair. *Science Adv.* Jun 2;7(23):eabd9371. doi: 10.1126/sciadv.abd9371., 2021

7. Michael J Soth, Kang Le, Maria Emilia Di Francesco, Matthew M Hamilton, Gang Liu, Jason P Burke, Chris L Carroll, Jeffrey J Kovacs, Jennifer P Bardenhagen, Christopher A Bristow, Mario Cardozo, Barbara Czako, Elisa de Stanchina, Ningping Feng, Jill R Garvey, Jason P Gay, Mary K Geck Do, Jennifer Greer, Michelle Han, Angela Harris, Zachary Herrera, Sha Huang, Virginia Giuliani, Yongying Jiang, Sarah B Johnson, Troy A Johnson, Zhijun Kang, Paul G Leonard, Zhen Liu, Timothy McAfoos, Meredith Miller, Pietro Morlacchi, Robert A Mullinax, Wylie S Palmer, Jihai Pang, Norma Rogers, Charles M Rudin, Hannah E Shepard, Nakia D Spencer, Jay Theroff, Qi Wu, Alan Xu, Ju Anne Yau, Giulio Draetta, **Carlo Toniatti**, Timothy P Heffernan, Philip Jones. Discovery of IPN60090, a Clinical Stage Selective Glutaminase-1 (GLS-1) Inhibitor with Excellent Pharmacokinetic and Physicochemical Properties. *J. Med. Chem.* 63(21): 12957-12977, 2020.
8. Sun Y, Bandi M, Lofton T, Smith M, Bristow CA, Carugo A, Rogers N, Leonard P, Chang Q, Mullinax R, Han J, Shi X, Seth S, Meyers BA, Miller M, Miao L, Ma X, Feng N, Giuliani V, Geck Do M, Czako B, Palmer WS, Mseeh F, Asara JM, Jiang Y, Morlacchi P, Zhao S, Peoples M, Tieu TN, Warmoes MO, Lorenzi PL, Muller FL, DePinho RA, Draetta GF, **Toniatti C**, Jones P, Heffernan TP, Marszalek JR. Functional Genomics Reveals Synthetic Lethality between Phosphogluconate Dehydrogenase and Oxidative Phosphorylation. *Cell Rep.* 8;26:469-482, 2019.
9. Jennifer R. Molina, Yuting Sun, Marina Protopopova, Sonal Gera, Madhavi Bandi, Timothy McAfoos, Pietro Morlacchi, Jennifer Bardenhagen, Christopher Bristow, Guang Gao, John Asara, Christopher Carroll, Edward Chang, Jason Cross, Barbara Czako, Angela Deem, Naval Daver, Ningping Feng, Jason Gay, Mary Geck Do, Jennifer Gree, Jing Han, Ahmed-Noor A. Agip, Judy Hirst, Sha Huang, Yongying Jiang, Zhijun Kang, Sergej Konoplev, Gang Liu, Timothy Lofton, Helen Ma, Polina Matre, Florian Muller, Robert Mullinax, Michael Peoples, Alessia Petrocchi, Jaime Rodriguez-Canale, Riccardo Serreli, Thomas Shi, Melinda Smith, Jay Theroff, Stefano Tiziani, Quanyun Xu, Stefan Ciurea, Gheath Al-Atrash, Jian-Wen Dong, John Frederick de Groot, Verlene K Henry, Caroline C Carrillo, Qi Zhang, **Carlo Toniatti**, Timothy P. Heffernan, Giulio F. Draetta, Marina Konopleva, Philip Jones, M. Emilia Di Francesco and Joseph R. Marszalek. An inhibitor of Oxidative phosphorylation exploits cancer vulnerability. *Nat Med.* 24:1036-1046, 2018.
10. Hong Jiang, Yisel Rivera-Molina, Karen Clise-Dwyer, Laura Bover, Luis Vence, Ying Yuan, Frederick Lang, **Carlo Toniatti**, Mohammad Hossain, Candelaria Gomez-Manzano, and Juan Fueyo-Margareto. Oncolytic adenovirus and tumor-targeting immune modulatory therapy improve autologous cancer vaccination. *Cancer Res.*, 77:3894-3907, 2017.
11. Michael Lee, Timothy Helms, Ningping Feng, Jason Gay, Qing Chang, Feng Tian, Ji Wu, **Carlo Toniatti**, Timothy Heffernan, Garth Powis, Lawrence Kwong, and E. Kopetz. Neuropilin-1 mediates Neutrophil Elastase uptake and cross-presentation in breast cancer cells. *J. Biol. Chem.*, 292(24):10295-10305.
12. Li L, Karanika S, Yang G, Wang J, Park S, Broom BM, Manyam GC, Wu W, Luo Y, Basourakos S, Song JH, Gallick GE, Karantanos T, Korentzelos D, Azad AK, Kim J, Corn PG, Aparicio AM, Logothetis CJ, Troncoso P, Heffernan T, **Toniatti C**, Lee HS, Lee JS, Zuo X, Chang W, Yin J, Thompson TC. Androgen receptor inhibitor-induced "BRCAness" and PARP inhibition are synthetically lethal for castration-resistant prostate cancer. *Sci. Signal.* May 23;10, 2017.
13. Genovese G, Carugo A, Tepper J, Robinson FS, Li L, Svelto M, Nezi L, Corti D, Minelli R, Pettazzoni P, Gutschner T, Wu CC, Seth S, Akdemir KC, Leo E, Amin S,

- Molin MD, Ying H, Kwong LN, Colla S, Takahashi K, Ghosh P, Giuliani V, Muller F, Dey P, Jiang S, Garvey J, Liu CG, Zhang J, Heffernan TP, **Toniatti C**, Fleming JB, Goggins MG, Wood LD, Sgambato A, Agaimy A, Maitra A, Roberts CW, Wang H, Viale A, DePinho RA, Draetta GF, Chin L. Synthetic vulnerabilities in mesenchymal sub-populations of pancreatic cancer cells undergoing clonal selection. *Nature*, 542:362-366, 2017.
14. Lee MS, Helms TL, Feng N, Gay J, Chang QE, Tian F, Wu JY, **Toniatti C**, Heffernan TP, Powis G, Kwong LN, Kopetz S. Efficacy of the combination of MEK and CDK4/6 inhibitors in vitro and in vivo in KRAS mutant colorectal cancer models. *Oncotarget*, 7:39595-39608, 2016.
 15. Petrocchi A, Leo E, Reyna NJ, Hamilton MM, Shi X, Parker CA, Mseeh F, Bardenhagen JP, Leonard P, Cross JB, Huang S, Jiang Y, Cardozo M, Draetta G, Marszalek JR, **Toniatti C**, Jones P, Lewis RT. Identification of potent and selective MTH1 inhibitors. *Bioorg Med Chem Lett*. 15:1503-7, 2016.
 16. Zhan Y, Kost-Alimova M, Shi X, Leo E, Bardenhagen JP, Shepard HE, Appikonda S, Vangamudi B, Zhao S, Tieu TN, Jiang S, Heffernan TP, Marszalek JR, **Toniatti C**, Draetta G, Tyler J, Barton M, Jones P, Palmer WS, Geck Do MK, Andersen JN. Development of novel cellular histone-binding and chromatin-displacement assays for bromodomain drug discovery. *Epigenetics Chromatin*., 8:37, 2015.
 17. Palmer WS, Poncet-Montange G, Liu G, Petrocchi A, Reyna N, Subramanian G, Theroff J, Yau A, Kost-Alimova M, Bardenhagen JP, Leo E, Shepard HE, Tieu TN, Shi X, Zhan Y, Zhao S, Barton MC1, Draetta G, **Toniatti C**, Jones P, Geck Do M, Andersen JN. Structure-Guided Design of IACS-9571, a Selective High-Affinity Dual TRIM24-BRPF1 Bromodomain Inhibitor. *J. Med. Chem.*, 59:1440-54, 2016.
 18. Bhavatarini Vangamudi, Thomas A. Paul, Parantu K. Shah, Maria Kost-Alimova, Lisa Nottebaum, Xi Shi, Yanai Zhan, Elisabetta Leo, Harshad S. Mahadeshwar, Alexei Protopopov, Andrew Futreal, Trang N. Tieu, Mike Peoples, Timothy P. Heffernan, Joseph R. Marszalek, **Carlo Toniatti**, Alessia Petrocchi, Dominique Verhelle, Dafydd R. Owen, Giulio Draetta, Philip Jones, Wylie S. Palmer, Shikhar Sharma, Jannik N. Andersen. The SMARCA2/4 ATPase Domain Surpasses the Bromodomain as a Drug Target in SWI/SNF-Mutant Cancers: Insights from cDNA Rescue and PFI-3 Inhibitor Studies. *Cancer Res.*, 75:3865-78, 2015
 19. Ferdinandos Skoulidis, Lauren Byers, Lixia Diao, Vassiliki Papadimitrakopoulou, Pan Tong, Julie Izzo, Carmen Behrens, Humam Kadara, Edwin Parra, Jaime Rodriguez-Canales, Jianjun Zhang, Uma Giri, Jayanthi Gudikote, Maria Cortez, Chao Yang, You-Hong Fan, Michael Peyton, Luc Girard, Kevin Coombes, **Carlo Toniatti**, Timothy Heffernan, Murim Choi, Garrett Frampton, Vincent Miller, John Weinstein, Roy Herbst, Kwok-Kin Wong, Jianhua Zhang, Padmanee Sharma, Gordon Mills, Waun Ki Hong, John Minna, James Allison, Andrew Futreal, Jing Wang, Ignacio Wistuba, and John Heymach. Co-occurring genomic alterations define major subsets of KRAS - mutant lung adenocarcinoma with distinct biology, immune profiles and therapeutic vulnerabilities. *Cancer Discovery*, 8:860-877, 2015.
 20. S.M.G. Williams, A.S. Kuznicki, P. Andrade, B. M. Dolinski, C. Elbi, R. O'Hagan, **C. Toniatti**. Treatment with the PARP inhibitor, niraparib, sensitizes colorectal cancer cell lines to irinotecan regardless of MSI/MSS status. *Cancer Cell International*, 15:14, 2015.
 21. Philip Jones, Keith Wilcoxon, Michael Rowley and **Carlo Toniatti**. Niraparib: a PARP inhibitor for the treatment of tumors with defective homologous recombination. *J. Med. Chem.*, 58:3302-3314, 2015.
 22. Piergiorgio Pettazoni, Andrea Viale, Parantu Shah, Alessandro Carugo, Haoqiang Ying, Huamin Wang, Giannicola Genovese, Sahil Seth, Rosalba Minelli, Tessa

- Green, Emmet Huang-Hobbs, Denise Corti, Nora Sanchez, Luigi Nezi, Matteo Marchesini, Avnish Kapoor, Wantong Yao, Maria Emilia Di Francesco, Alessia Petrocchi, Angela K. Deem, Kenneth Scott, Simona Colla, Gordon B. Mills, Jason B. Fleming, Timothy P. Heffernan, Philip Jones, **Carlo Toniatti**, Ronald A. DePinho, Giulio F. Draetta. Genetic events that limit the efficacy of MEK and RTK inhibitor therapies in a mouse model of KRAS-driven pancreatic cancer. *Cancer Res.*, 75:1091-101, 2015.
23. **Toniatti C.**, Jones P., Graham H., Bruno Pagliara B, Giulio Draetta. Oncology Drug Discovery: Planning a Turnaround. *Cancer Discov.*, 4:397-404, 2014.
 24. Bridges KA, **Toniatti C.**, Buser CA, Liu H, Buchholz TA, Meyn RE. Niraparib (MK-4827), a novel poly(ADP-Ribose) polymerase inhibitor, radiosensitizes human lung and breast cancer cells. *Oncotarget*, 5:5076-86, 2014.
 25. Sandhu SK, Schelman WR., Wilding G, Moreno V, Baird RD, Miranda S, Hylands L, Riisnaes R, Forster M, Omlin A, Kreischer N, Thway J, Gevensleben H, Sun L, Loughney J, Chatterjee M, **Toniatti C.**, Carpenter CL, Iannone R, Kaye SB, de-Bono JS, Wenham RM. First in Human Trial of MK4827, a Poly(ADP-ribose) Polymerase (PARP) Inhibitor with Antitumour Activity in BRCA Mutation Carrier and Sporadic Cancer Patients. *Lancet Oncol.* 14:882-92, 2013
 26. Guertin AD, Li J, Liu Y, Hurd MS, Schuller AG, Long B, Hirsch HA, Feldman I, Benita Y, **Toniatti C.**, Zawel L, Fawell SE, Gilliland G, Shumway SD. Preclinical Evaluation of the WEE1 Inhibitor MK-1775 as Single Agent Anticancer Therapy. *Mol. Cancer. Ther.* 12:1442-52, 2013
 27. Yuan J, Strack PR, **Toniatti C.**, Pelletier M. A zinc finger nuclease assay to rapidly quantitate homologous recombination proficiency in human cell lines. *Anal. Biochem.* 434:96-8, 2013.
 28. Aarts M, Sharpe R, Garcia-Murillas I, Gevensleben H, Hurd MS, Shumway SD, Toniatti C, Ashworth A, Turner NC. Forced mitotic entry of S-phase cells as a therapeutic strategy induced by inhibition of WEE1. *Cancer Discov.* 2:524-39, 2012
 29. Guertin AD, Martin MM, Roberts B, Hurd M, Qu X, Miselis NR, Liu Y, Li J, Feldman I, Benita Y, Bloecher A, **Toniatti C.**, Shumway SD. Unique functions of CHK1 and WEE1 underlie synergistic anti-tumor activity upon pharmacologic inhibition. *Cancer Cell Int.* 12:45 (2012)
 30. Stec EM, Rachel Needham R., Warrener P., Palmieri A., Bradshaw JD, Kim S., Ferrer M., Chung N., Santini F., Strulovici B., **Toniatti C.**, Jackson A.L., Takagi T., Dai H., Bartz S.R., Cleary M.A., Carleton M, Linsley P.S. Expanding the BRCA network of DNA repair genes using a genome-scale siRNA screen for enhancers of poly(ADP-ribose) polymerase inhibitors. *J. Biomol. Screen.* 17:1316-28 (2012)
 31. Warrener P., Kim S., Biery M., **Toniatti C.**, Cleary M.A., Linsley P.S., Carleton M. Synthetic lethality of PARP inhibition in BRCA-network disrupted tumor cells is associated with interferon pathway activation and enhanced by interferon-gamma. *Apoptosis*, 17:691-701 (2012)
 32. Wang L., Mason, KA., Ang K., Buchholz T., Valdekanas D., Anjili Mathur, Buser-Doepner C., **Toniatti C.**, Milas L. MK-4827, a PARP-1/-2 inhibitor, strongly enhances response of human lung and breast cancer xenografts to radiation. *Invest New Drugs.* 30:2113-20, 2012.
 33. Dharmapuri S, Peruzzi D, Marra E, Palombo F, Bett AJ, Bartz SR, Yong M, Ciliberto G, La Monica N, Buser CA, **Toniatti C.**, Aurisicchio L. Intratumor RNA interference of cell cycle genes slows down tumor progression. *Gene Ther.* 2011 Jul;18(7):727-33 (2011).
 34. Pescatore, G.; Branca, D.; Fiore, F.; Kinzel, O.; Llauger Bufi, L.; Muraglia, E.; Orvieto, F.; Rowley, M.; **Toniatti, C.**; Torrisi, C.; Jones, P. Identification and SAR

- of Novel Pyrrolo[1,2-a]pyrazin-1(2H)-one Derivatives as Inhibitors of Poly(ADP-ribose) polymerase-1 (PARP-1). *Bioorg. Med. Chem. Lett.* 20, 1094-1099 (2010).
35. Ferrigno, F.; Branca, D.; Kinzel, O.; Lillini, S.; Llauger Bufi, L.; Monteagudo, E.; Muraglia, E.; Rowley, M.; Schultz-Fademrecht, C.; **Toniatti, C.**; Torrissi, C.; Jones, P. Development of substituted 6-[4-fluoro-3-(piperazin-1-ylcarbonyl)benzyl]-4,5-dimethylpyridazin-3(2H)-ones as potent poly(ADP-ribose) polymerase-1 (PARP-1) inhibitors active in BRCA deficient cells. *Bioorg. Med. Chem. Lett.* 20, 1100-1105 (2010).
 36. Torrissi, C.; Bisbocci, M.; Ingenito, R.; Ontoria, J. M.; Rowley, M.; Schultz-Fademrecht, C.; **Toniatti, C.**; Jones, P. Discovery and SAR of novel, potent and selective hexahydrobenzonaphthyridinone inhibitors of poly(ADP-ribose)polymerase-1 (PARP-1). *Bioorg. Med. Chem. Lett.* 20, 448-452 (2010).
 37. Scarpelli, R.; Boueres, J. K.; Cerretani, M.; Ferrigno, F.; Ontoria, J. M.; Rowley, M.; Schultz-Fademrecht, C.; **Toniatti, C.**; Jones, P. Synthesis and biological evaluation of substituted 2-phenyl-2H-indazole-7-carboxamides as potent poly(ADP-ribose) polymerase (PARP) inhibitors. *Bioorg. Med. Chem. Lett.* 20, 488-492 (2010).
 38. Jones, P.; Altamura, S.; Boueres, J.; Ferrigno, F.; Fonsi, M.; Giomini, C.; Lamartina, S.; Monteagudo, E.; Ontoria, J. M.; Orsale, M. V.; Palumbi, M. C.; Pesci, S.; Roscilli, G.; Scarpelli, R.; Schultz-Fademrecht, C.; **Toniatti, C.**; Rowley, M. Discovery of 2-{4-[(3S)-Piperidin-3-yl]phenyl}-2H-indazole-7-carboxamide (MK-4827): A Novel Oral Poly(ADP-ribose)polymerase (PARP) Inhibitor Efficacious in BRCA-1 and -2 Mutant Tumors. *J. Med. Chem.* 52, 7170-85 (2009).
 39. Branca D, Cerretani M, Jones P, Koch U, Orvieto F, Palumbi MC, Rowley M, **Toniatti C.**, Muraglia E. Identification of aminoethyl pyrrolo dihydroisoquinolinones as novel poly(ADP-ribose) polymerase-1 inhibitors. *Bioorg Med Chem Lett.* 2009 Aug 1;19(15):4042-5.
 40. Orvieto, F., Branca, D., Giomini, C., Jones, P., Koch, U., Ontoria, J. M., Palumbi, M. C., Rowley, M., **Toniatti C.**, Muraglia, E. Identification of substituted pyrazolo[1,5-a]quinazolin-5(4H)-one as potent poly(ADP-ribose)polymerase-1 (PARP-1) inhibitors. *Bioorg. Med. Chem. Lett.*, **19**, 4196-4200 (2009).
 41. Lamartina S, Cimino M, Roscilli G, Dammassa E, Lazzaro D, Rota R, Ciliberto G, **Toniatti C.** Helper-dependent adenovirus for the gene therapy of proliferative retinopathies: stable gene transfer, regulated gene expression and therapeutic efficacy. *J Gene Med.* 9: 862-74 (2007)
 42. **Toniatti C.** and Steinkühler C. Struttura della cromatina e modificazioni epigenetiche in: *Argomenti di Biologia Molecolare*, ed. G. Melino and G. Ciliberto, Società editrice Universo, Chapter 5, 79-108 (2006)
 43. Kinzel O., Fattori D., Muraglia E., Gallinari P, Nardi MC, Paolini C, Roscilli G, **Toniatti C.**, Gonzalez Paz O, Laufer R, Lahm A, Tramontano A, Cortese R, De Francesco R, Ciliberto G, Koch U: A structure-guided approach to an orthogonal estrogen-receptor-based gene switch activated by ligands suitable for in vivo studies. *J Med Chem.* 49:5404-5407 (2006).
 44. Gallinari P, Lahm A, Koch U, Paolini C, Nardi MC, Roscilli G, Kinzel O, Fattori D, Muraglia E, **Toniatti C.**, Cortese R, De Francesco R, Ciliberto G : A functionally orthogonal estrogen receptor-based transcription switch specifically induced by a nonsteroid synthetic ligand. *Chem Biol.* 12 :883-93 (2005).
 45. Kinose F, Roscilli G, Lamartina S, Anderson KD, Bonelli F, Spence SG, Ciliberto G, Vogt TF, Holder DJ, **Toniatti C.**, Thut CJ: Inhibition of retinal and choroidal neovascularization by a novel KDR kinase inhibitor. *Mol Vis.* 27, 11:366-73 (2005)
 46. **Toniatti C.**, Bujard H., Cortese C. and Ciliberto G.: Gene Therapy Progress and Prospects: Transcription Regulatory Systems. *Gene Ther.*,11, 649-657 (2004).

47. **Toniatti C.**, Palombo F., Cortese R. and Ciliberto G.: Ligand-dependent transcription switches and their potential for gene therapy in: *Gene and Cell Therapy: Therapeutic Mechanisms and Strategies*, ed. N.S. Templeton, Marcel Dekker, Chapter 22, 413-447 (2004).
48. Fattori E., La Monica N., Ciliberto G. and **Toniatti C.**: Electro-gene-transfer: a new approach for muscle gene delivery, in: "*Synthetic DNA Delivery Systems*", ed. Dan Luo and M. Saltzman, Landes Biosciences (2003).
49. Lamartina S., Silvi L., Roscilli G., Casimiro D., Simon AJ, Davies M-E, Shiver J.W., Rinaudo C.D., Zampaglione I., Fattori E., Colloca S., Gonzalez Paz O., Laufer R., Bujard H., Cortese R., Ciliberto G. and **Toniatti C.**: Construction of an rtTA2^s-M2/tTS^{kid}-based transcription regulatory switch which displays no basal activity, great inducibility and high responsiveness to Doxycycline in mice and non-human primates. *Mol. Ther.* 7, 271-280 (2003).
50. Fattori E., La Monica N., Ciliberto G. and **Toniatti C.**: Electro-gene-transfer: a new approach for muscle gene delivery. *Som. Cell and Mol. Gen.* 2, 75-84 (2002).
51. Roscilli G., Rinaudo C.D., Cimino M., Sporeno E., Lamartina S., Ciliberto G. and **Toniatti C.**: Long-term and tight control of gene expression in mouse skeletal muscle by a new hybrid human transcription factor. *Mol. Ther.*, 6, 653-663 (2002).
52. Salucci V, Scarito A, Aurisicchio L, Lamartina S, Nicolaus G, Giampaoli S, Gonzalez-Paz O, **Toniatti C.**, Bujard H, Hillen W, Ciliberto G and Palombo F: Tight control of gene expression by a helper-dependent adenovirus vector carrying the rtTA2^s-M2 tetracycline transactivator and repressor system. *Gene Ther.* 9, 1415-1421 (2002).
53. Lamartina S., Roscilli G., Rinaudo C.D., Sporeno E., Silvi L., Hillen W., Bujard H., Cortese R., Ciliberto G. and **Toniatti C.**: Stringent control of gene expression in vivo by using novel doxycycline-dependent transactivators. *Hum. Gene Ther.*, 13, 199-210 (2002).
54. Samakoglu, S., Fattori E., Lamartina S., **Toniatti C.**, Stockholm D., Heard J. M. and Bohl D.: Beta-minor Globin mRNA accumulation in reticulocytes governs improved erythropoiesis in beta-thalassemic mice after erythropoietin cDNA electro-transfer in muscles. *Blood* 97, 2213-2220 (2001).
55. Lamartina S., Ciliberto G. and **Toniatti C.**: Selective cleavage of AAVS1 substrates by Adenoassociated virus (AAV) type 2 Rep68 protein is dependent on topological and sequence constraints. *J. Virol.* 74:8831-8842 (2000).
56. Rinaudo, D. and **Toniatti C.**: A sensitive ELISA for mouse erythropoietin. *Biotechniques* 29:218-220 (2000).
57. Lamartina, S., Sporeno, E., Fattori, E. and **Toniatti C.**: Characteristics of the Adeno-associated virus pre-integration site in human chromosome 19: open chromatin conformation and transcription competent environment. *J. Virol.* 74, 7671-7677 (2000).
58. Rinaudo, D., Lamartina, S., Roscilli, G., Ciliberto, G. and **Toniatti C.**: Conditional site-specific integration into human chromosome 19 by using a ligand-dependent chimeric Adeno-associated virus/Rep protein. *J. Virol.*, 74, 281-294 (2000).
59. Recchia A., Parks R.J., Lamartina S., **Toniatti C.**, Pieroni L., Palombo F., Ciliberto G., Graham F. L., Cortese R., Ciliberto G., La Monica N. and Colloca S.: Site-specific integration mediated by a hybrid adenovirus/adenoassociated virus vector. *Proc. Natl. Acad. Sci. USA*, 96, 2615-2620 (1999).
60. Frasca D., Doria G., Barattini P., Guidi F., Salvati A.L., **Toniatti C.** and Ciliberto G.: Activation of gp130 signaling in vivo by the IL-6 super-agonist K-7/D-6 accelerates repopulation of lymphoid organs after irradiation. *Eur. J. Immunol.*, 29:300-310 (1999).

61. Borsellino N., Bonavida B., Ciliberto G., **Toniatti C.**, Travali S. and D'Alessandro N.: Interleukin-6, oncostatin M and gp130 subunit are drug resistance factors in hormone-independent prostate cancer cells. *Cancer*, 85: 134-144 (1999).
62. Lamartina S., Roscilli G., Rinaudo D., Delmastro P. and **Toniatti C.**: Lipofection of purified Adeno-associated virus Rep68 protein: toward a chromosome targeting non-viral particle. *J. Virol.*, 72, 7653-7658 (1998).
63. Ciliberto G., Lahm A., Paonessa G., Savino R. and **Toniatti C.**: Designing new agonists/antagonists of growth factor receptors - The rational design of a super-antagonist of the IL-6 receptor in: *Growth Factors and Receptors: a practical approach* (ed. I. A. McKay and K.D. Brown), chapter 3, p. 51-81, IRL press, Oxford (1998).
64. Romano M., Sironi M., **Toniatti C.**, Polentarutti N., Fruscella P., Ghezzi P., Faggioni R., Luini W., Sozzani S., Bussolino F., Poli V., Ciliberto G. and Mantovani A.: IL-6, in concert with soluble IL-6 receptor, selectively induces chemokine production in endothelial cells and participates in leukocyte recruitment in vivo. *Immunity*, 6, 315 – 325 (1997).
65. Savino R., Demartis A., Ciapponi L., Sporeno E., **Toniatti C.**, Bernassola F., Melino G., Klein B. and Ciliberto G.: The receptor superantagonist Sant7: a potent and safe inhibitor of IL-6 on human myeloma cells. *Oncology Reports*, 4, 485-492 (1997).
66. Di Santo E., Poli V., Fattori E., Alonzi T., **Toniatti C.**, Sironi M., Ricciardi-Castagnoli P. and Ghezzi P.: Differential effect of IL-6 on systemic and central production of TNF: a study with IL-6 deficient mice. *Cytokine* 9, 300-306 (1997).
67. Giraud E., Arese M. **Toniatti C.**, Strasly M., Primo L., Mantovani A., Ciliberto G. and Bussolino F.: IL-6 is an in vitro and in vivo autocrine growth factor for middle T antigen-transformed endothelial cells. *J. Immunol.*, 157, 2168-2623 (1996).
68. Sporeno E., Savino R., Ciapponi L., Paonessa G., Cabibbo A., Lahm A., Pulkki K., Ren-Xiao S., **Toniatti C.**, Klein B. and Ciliberto G.: Human IL-6 receptor super-antagonists with high potency and wide spectrum on multiple myeloma. *Blood*, 87, 4510-4519 (1996).
69. **Toniatti C.**, Cabibbo A., Sporeno E., Salvati A.L., Cerretani M., Serafini S., Lahm A., Cortese R. and Ciliberto G.: Engineering human interleukin-6 to obtain variants with strongly enhanced bioactivity. *EMBO J.*, 15, 2726-2737 (1996).
70. Romani L., Mencacci A., Cenci E., Spaccapelo R., **Toniatti C.**, Puccetti P., Bistoni F. and Poli V.: Impaired neutrophil response and CD4(+) T helper cell development in interleukin 6-deficient mice infected with *Candida albicans*. *J. Exp. Medicine*, 183, 1345-1355 (1996).
71. Chai Z., Gatti S., **Toniatti C.**, Poli V. and Bartfai T.: Interleukin (IL)-6 gene expression in the central nervous system is necessary for fever response to lipopolysaccharide or IL-1 beta: a study on IL-6 deficient mice. *J. Exp. Medicine*, 183, 311-316 (1996).
72. Martin F., **Toniatti C.**, Salvati A. L., Ciliberto G., Cortese R. and Sollazzo M.: Coupling protein design and in vitro selection strategies: improving specificity and affinity of a designed beta-protein IL-6 antagonist. *J. Mol. Biol.*, 255, 86-97 (1996).
73. Cabibbo A., Sporeno E., **Toniatti C.**, Altamura S., Savino R., Paonessa G. and Ciliberto G.: Monovalent phage display of human Interleukin(hIL)-6: selection of superbinder variants from a complex molecular repertoire in hIL-6 D-helix. *Gene* 167, 41-47 (1995).
74. Silvani A., Ferrari G., Paonessa G., **Toniatti C.**, Parmiani G. and Colombo M.P.: Down regulation of interleukin 6 receptor alpha chain in interleukin 6 transduced melanoma cells causes selective resistance to IL-6 but not to Oncostatin M. *Cancer Res.* 55, 2200-2205 (1995).

75. Salvati A. L., Lahm A., Paonessa G., Ciliberto G. and **Toniatti C.**: Interleukin-6 (IL-6) antagonism by IL-6 receptor alpha mutated in the predicted gp130 binding interface. *J. Biol.Chem.* 270, 12242-12249 (1995).
76. Paonessa G., Graziani R., De Serio A., Savino R., Ciapponi L., Lahm A., Salvati A. L., **Toniatti, C.** and Ciliberto, G.: Two distinct and independent sites on IL-6 trigger gp130 dimer formation and signalling. *EMBO J.* 14, 1942-1951 (1995).
77. Lahm, A., Savino, R., A. L. Salvati, Cabibbo, A., Ciapponi, L., Demartis, A., **Toniatti, C.**, Paonessa, G., Altamura, S. and Ciliberto, G.: The molecular design of human IL-6 receptor antagonists. *Ann. N. Y. Acad. Sci.*, 762, 136-151 (1995).
78. Savino R., Ciapponi L., Lahm A., Demartis A., Cabibbo A., **Toniatti C.**, Delmastro P., Altamura S. and Ciliberto G.: Rational design of a receptor super-antagonist of human interleukin-6. *EMBO J.* 13, 5863-5870 (1994).
79. Martin F., **Toniatti C.**, Salvati A. L., Venturini S., Ciliberto G., Cortese R. and Sollazzo M.: The affinity-selection of a minibody polypeptide inhibitor of human interleukin-6. *EMBO J.* 13, 5303-5309 (1994).
80. Savino R., Lahm A., Salvati A.L., Ciapponi L., Sporeno E., Altamura S., Paonessa G., **Toniatti C.** and Ciliberto G.: Generation of IL-6 antagonists by substituting residues in helix A, chapter 8, pages 93-98 in: *Cytokines: Basic Principles and Practical Applications, Challenges of Modern Medicine, vol.8, S. Romagnani, G. Del Prete and A.K. Abbas, Eds., Ares-Serono Symposia Publications* (1994).
81. Piaggio G., Tomei L., **Toniatti C.**, De Francesco R., Gerstner J. and Cortese R.: LFB1/HNF-1 acts as a repressor of its own transcription. *Nucleic Acids Res.* 22, 4284-4290 (1994).
82. Sporeno E., Paonessa G., Salvati A.L., Graziani R., Delmastro P., Ciliberto G. and **Toniatti C.**: Oncostatin M binds directly to gp130 and behaves as interleukin-6 antagonist on a cell line expressing gp130 but lacking functional oncostatin M receptors. *J. Biol. Chem.* 269, 10991-10995 (1994).
83. Savino R., Lahm A., Salvati A.L., Ciapponi L., Sporeno E., Altamura S., Paonessa G., **Toniatti C.** and Ciliberto G.: Generation of interleukin-6 receptor antagonists by molecular-modeling guided mutagenesis of residues important for gp130 activation. *EMBO J.* 13, 1357-1367 (1994).
84. Wallace A., Altamura S., **Toniatti C.**, Vitelli A., Bianchi E., Delmastro P., Ciliberto G. and Pessi A : A Multimeric Synthetic Combinatorial Peptide Library. *Peptide Res.* 7, 27-31 (1994).
85. Ciliberto G., Colantuoni V., De Francesco R., De Simone E., Monaci P., Nicosia A., Ramji D.P., **Toniatti C.** and Cortese R.: Transcriptional control of Gene Expression in Hepatic Cells, chapter 7, pages 162-242, in: *Gene Expression: General and Tissue Specific, Michael Karin, Ed., Birkhauser, Boston* (1993).
86. **Toniatti C.**, Monaci P., Nicosia A., Cortese R. and Ciliberto G.: A bipartite activation domain is responsible for the activity of transcription factor HNF-1/LFB1 in cells of hepatic and non-hepatic origin. *DNA & Cell. Biol.* 12, 199-208 (1993).
87. Fiorillo M.T., **Toniatti C.**, Van Snick J. and Ciliberto G.: Expression of the murine interleukin 6 receptor in hepatoma cells: the intracytoplasmic domain is not required for signal transduction. *Eur. J. Immunol.* 22, 799-804 (1992).
88. **Toniatti C.**, Demartis A., Monaci P., Nicosia A. and Ciliberto G.: Synergistic trans-activation of the human C-reactive protein promoter by transcription factor HNF-1 binding at two distinct sites. *EMBO J.* 9, 4467-4475 (1990).
89. **Toniatti C.**, Arcone R., Majello B., Ganter U., Arpaia G. and Ciliberto G.: Regulation of the human C-reactive protein gene, a major marker of inflammation and cancer. *Mol. Biol. Med.* 7, 199-212 (1990).

90. Majello B., Arcone R., **Toniatti C.** and Ciliberto G.: Constitutive and IL-6 induced nuclear factors that interact with the human C-reactive protein promoter. *EMBO J.* 9, 457-465 (1990).
91. Ganter U., Arcone R., **Toniatti C.**, Morrone G. and Ciliberto G.: Dual control of C-reactive protein gene expression by interleukin-1 and interleukin-6. *EMBO J.* 8, 3773-3779 (1989).
92. Giuditta A., Perrone Capano C., D' Onofrio G., **Toniatti C.**, Menna T. and Hydén H.: Synthesis of rat brain DNA during acquisition of an appetitive task. *Pharmacol. Biochem. Behav.* 25, 651-658 (1986)

PATENTS

- 1) Ciliberto G., Lahm A., **Toniatti C.**, and Savino R.: A Methodology For Selecting Superagonists, Antagonists and Superantagonists of Human Interleukin-6 Based on Receptor Complex Three Dimensional Modelling. WO 1996/18648.
- 2) Ciliberto G. and **Toniatti C.**: Interleukin-6 (IL-6) antagonists. WO 1996/17869.
- 3) Ciliberto G., Savino R., **Toniatti C.**, Borsellino N.: Anti-Tumour Pharmaceutical Compositions Capable of Reducing Drug Resistance in Tumour Cells. WO 1998/58674.
- 4) **Toniatti C.**, Ciliberto G. and Rinaudo C.: Hormone-Dependent Forms of Adeno Associated Virus (AAV) Rep Proteins. WO 1999/27110.
- 5) **Toniatti C.**, Ciliberto G. and Cortese R.: Methods and Means for regulation of gene expression. WO 2001/98506.
- 6) Ciliberto G., De Francesco R., Fattori D., Gallinari P., Kinzel O. D., Koch U., Muraglia E., **Toniatti C.**, Cortese R., Lahm A. Orthogonal Gene Switches WO 2005/040212
- 7) Timothy Heffernan, **Carlo Toniatti**, Jeffrey Kovacs, Virginia Giuliani, Nakia Spencer, Maria Emilia Di Francesco, Christopher A. Bristow. Glutaminase inhibitor therapy. WO2016/004418
- 8) Michael A. Curran, Ashvin R. Jaiswal, Dongxing Zha, Kui Voo, **Carlo Toniatti**, Bianka Prinz, Nga Rewa, Eric Krauland. Dual specificity antibodies to PD-L1 and PD-L2 and methods of use therefor. WO2019/182896
- 9) Michael A. Curran, **Carlo Toniatti**, Ashvin R. Jaiswal, Dongxing Zha, Kui Voo, Bianka Prinz, Nadthakam Boland. Human PD-L1 antibodies and methods of use therefor. WO2019/075097
- 10) Michael A. Curran, Ashvin R. Jaiswal, Dongxing Zha, Kui Voo, **Carlo Toniatti**, Bianka Prinz, Nga Rewa, Eric Krauland Human PD-L2 antibodies and methods of use therefor. WO2019/182896

SELECTED CONFERENCE PRESENTATIONS

1. Jennifer Molina, Madhavi Bandi, Jennifer Bardenhagen, Christopher Bristow, Christopher Carroll, Edward Chang, Jason Cross, Naval Daver, Ningping Feng, Jason Gay, Mary Geck Do, Jennifer Greer, Jing Han, Judy Hirst, Sha Huang, Yongying Jiang, Zhijun Kang, Marina Konopleva, Gang Liu, Helen Ma, Polina Matre, Timothy McAfoos, Funda Meric-Bernstam, Pietro Morlacchi, Florian Muller Marina Protopopova, Melinda Smith, Sonal Sonal, Yuting Sun, Jay Theroff, Andrea Viale, Quanyun Xu, **Carlo Toniatti**, Giulio Draetta, Philip Jones, M. Emilia Di Francesco, Joseph R. Marszalek. IACS-010759, a novel inhibitor of complex I in Phase I clinical development to target OXPHOS dependent tumors. AACR Annual Meeting, April 1-5, 2017, Washington DC.
2. **Carlo Toniatti**, Niranjan Yanamandra, Kui Voo, Amin Al-Shami, Laura Bover, Peter Morley, Sara Brett, Tim Lofton, Jennifer Greer, Ningping Feng,

- Ignacio Ivan Wistuba, Sabyasachi Bhattacharya, Christopher Hopson, David Kilian Heather Jackson, Paul Bojczuk, Mili Mandal, Junping Jing, Kevin French, Roopa Srinivasan, Axel Hoos. Engaging the immune system with GSK3174998, a potent anti-OX40 agonist antibody. AACR April 16-20, 2016; New Orleans, LA
3. Elisabetta Leo, Alessia Petrocchi, Jennifer Bardenhagen, Maria Alimova, Xi Shi, Connor Parker, Naphtali Reyna, Matthew Hamilton, Edward Felix, Andrzej Mazan, Christian Dillon, Faika Mseeh, Joseph R. Marszalek, **Carlo Toniatti**, Giulio Draetta, Phil Jones, Richard T. Lewis. Identification of potent, cell active MTH1 inhibitors and their use in target validation studies. AACR-NCI-EORTC, November 5-9, 2015, Boston, MA.
 4. **Carlo Toniatti**: Precision medicine: cancer genomics and beyond. Cancer Genomics, EMBL Conference. 1 - 4 Nov 2015, EMBL Heidelberg, Germany (Invited Speaker)
 5. Hong Jiang, Candelaria Gomez-Manzano, Karen Clise-Dwyer, Laura Bover, Luis M Vence, Frederick F Lang, Charles A Conrad, **Carlo Toniatti**, Frank Tufaro, Juan Fueyo. Delta-24-RGDOX: expressing OX40L in gliomas using replication competent oncolytic adenoviruses induces anti-tumor effect and anti-glioma immune memory. CRI-CIMT-EATI-AACR, September 16-19, 2015, New York, NY.

Autorizzo il trattamento dei dati personali contenuti nel mio curriculum vitae in base all'art. 13 del D. Lgs. 196/2003 e all'art. 13 del Regolamento UE 2016/679 relativo alla protezione delle persone fisiche con riguardo al trattamento dei dati personali

April 20th, 2022



Carlo Toniatti