### NAME: COUTROUPIS ELIZABETH DEPARTMENT: PHARMACOLOGY MATRIC NUMBER: 18/MHS07/013 COURSE: PHA 210

The field of Medical Biotechnology includes research and development of technology used in the medical, agricultural and pharmaceutical industries. Medical biotechnology is the use of living cells and cell materials to research and produce pharmaceutical and diagnostic products that help treat and prevent human diseases. Most medical biotechnologists work in academic or industrial settings. Medical biotechnology is the use of living cells and cell materials to research and produce pharmaceutical and diagnostic products that help treat and prevent human diseases. Most medical biotechnologists work in academic or industrial settings. In academic laboratories, these professionals conduct experiments as part of medical research studies; industrial biotechnologists work toward developing drugs or vaccines. The medical biotechnology field has helped bring to market microbial pesticides, insect-resistant crops, and environmental clean-up techniques. Although genomics and its applications (viz. gene therapy) commonly come to mind when medical biotechnology is mentioned, it should be remembered that other disciplines such as bioinformatics (see also Bioinformatics on Post Genomic Era- From Genomes to Systems Biology), nanotechnology (see also Nanomedicine and Nanorobotics), fermentation technology (see also Basic Strategies of Cell Metabolism) and cell technology (see also Microbial Cell Culture) also play an important role in the

There are different aspects of medical biotechnology: Pharmacology

Gene therapy Stem cells Tissue engineering Xeno -transplantation Vaccines Antibodies

## GENE THERAPY;

Gene therapy is an experimental technique that uses genes to treat or prevent disease. In the future, this technique may allow doctors to treat a disorder by inserting a gene into a patient's cells instead of using drugs or surgery. ... Inactivating, or "knocking out," a mutated gene that is functioning improperly.

Gene therapy (also called human gene transfer) is a medical field which focuses on the utilization of the therapeutic delivery of nucleic acid into a patient's cells as a drug to treat disease. The first attempt at modifying human DNA was performed in 1980 by Martin Cline, but the first successful nuclear gene transfer in humans, approved by the National Institutes of Health, was performed in May 1989. The first therapeutic use of gene transfer as well as the first direct insertion of human DNA into the nuclear genome was performed by French Anderson in a trial starting in September 1990. It is thought to be able to cure many genetic disorders or treat them over time.

Between 1989 and December 2018, over 2,900 clinical trials were conducted, with more than half of them in phase I.As of 2017, Spark Therapeutics' Luxturna (RPE65 mutation-induced blindness) and Novartis' Kymriah (Chimeric antigen receptor T cell therapy) are the FDA's first approved gene therapies to enter the market. Since that time, drugs such as Novartis' Zolgensma and Alnylam's Patisiran have also received FDA approval, in addition to other companies' gene therapy drugs. Most of these approaches utilize adeno-associated viruses (AAVs) and lentiviruses for performing gene insertions, *in vivo* and *ex vivo*, respectively. ASO / siRNA approaches such as those conducted

by Alnylam and Ionis Pharmaceuticals require non-viral delivery systems, and utilize alternative mechanisms for trafficking to liver cells by way of GalNAc transporters.

The introduction of CRISPR gene editing has opened new doors for its application and utilization in gene therapy. Solutions to medical hurdles, such as the eradication of latent human immunodeficiency virus (HIV) reservoirs and correction of the mutation that causes sickle cell disease, may soon become a tangible reality.

Not all medical procedures that introduce alterations to a patient's genetic makeup can be considered gene therapy. Bone marrow transplantation and organ transplants in general have been found to introduce foreign DNA into patients.

### XENO -TRANSPLANTATION;

Xenogeneic transplantation is defined as the transplantation of living xenogeneic cells, tissues or organs. The term additionally encompasses the in vivo use of human body fluids, cells, tissues or organs that have had ex vivo contact with these living xenogeneic materials. Xenogeneic transplantation has the potential to supplement the limited supply of human material for transplantation and may even become an alternative.

However, xenotransplantation presents specific immunological problems for recipients. It also may transmit pathogens from the animal source to the recipient, and subsequently to the general public. Immunosuppression, inadequate or non-existent diagnostic tools and an absence of effective therapy compound these risks. Ethical questions in xenotransplantation include whether potential recipients (and possibly their family and other close contacts) have given voluntary, informed consent. An additional concern is whether monitoring of recipients should be mandated along with containment measures when transmission of an animal pathogen is suspected. Other considerations relate to the ethics of the use of animals as sources of material for transplantation in humans.

In several countries, xenotransplantation is now featured in clinical research, and in some cases it is a part of medical practice. Xenotransplant experiments have been reported in countries that have no regulatory oversight. Moreover, "xenotransplant tourism" by patients who are willing to pay for unproven interventions in countries without adequate controls risks global dissemination of new pathogens and may undermine this fledgling field.

## TISSUE ENGINEERING;

Tissue engineering is the use of a combination of cells, engineering, and materials methods, and suitable biochemical and physicochemical factors to improve or replace biological tissues. Tissue engineering involves the use of a tissue scaffold for the formation of new viable tissue for a medical purpose. While it was once categorized as a sub-field of biomaterials, having grown in scope and importance it can be considered as a field in its own.

While most definitions of tissue engineering cover a broad range of applications, in practice the term is closely associated with applications that repair or replace portions of or whole tissues (i.e., bone, cartilage,] blood vessels, bladder, skin, muscle etc.). Often, the tissues involved require certain mechanical and structural properties for proper functioning. The term has also been applied to efforts to perform specific biochemical functions using cells within an artificially-created support system (e.g. an artificial pancreas, or a bio artificial liver). The term *regenerative medicine* is often used synonymously with tissue engineering, although those involved in regenerative medicine place more emphasis on the use of stem cells or progenitor cells to produce tissues.

# STEM CELLS

Multiple populations of stem cells have been indicated to potentially participate in regeneration of injured organs. Especially, embryonic stem cells (ESC) and recently inducible pluripotent stem cells (iPS) receive a marked attention from scientists and clinicians for regenerative medicine because of their high proliferative and differentiation capacities. Despite that ESC and iPS cells are expected to give rise into multiple regenerative applications when their side effects are overcame during appropriate preparation procedures, in fact their most recent application of human ESC may, however, reside in their use as a tool in drug development and disease modeling. This review focuses on the applications of stem cells in pharmaceutical biotechnology. We discuss possible relevance of pluripotent cell stem populations in developing physiological models for any human tissue cell type useful for pharmacological, metabolic and toxicity evaluation necessary in the earliest steps of drug development. The present models applied for preclinical drug testing consist of primary cells or immortalized cell lines that show limitations in terms of accessibility or relevance to their in vivo counterparts. The availability of renewable human cells with functional similarities to their in vivo counterparts is the first landmark for a new generation of cell-based assays. We discuss the approaches for using stem cells as valuable physiological targets of drug activity which may increase the strength of target validation and efficacy potentially resulting in introducing new safer remedies into clinical trials and the marketplace. Moreover, we discuss the possible applications of stem cells for elucidating mechanisms of disease pathogenesis. The knowledge about the mechanisms governing the development and progression of multitude disorders which would come from the cellular models established based on stem cells, may give rise to new therapeutical strategies for such diseases. All together, the applications of various cell types derived from patient specific pluripotent stem cells may lead to targeted drug and cellular therapies for certain individuals.